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Treatment Patterns and Responses in Adult Primary Immune Thrombocytopenia Patients: A Single-Centre Study

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Abstract

Objective: To characterize real-world treatment patterns and outcomes of adults with primary immune thrombocytopenia (ITP) treated at a tertiary care hospital.

Methodology: We retrospectively analyzed electronic medical records of 56 adult patients with primary ITP. Platelet count changes following treatment were assessed using the Wilcoxon signed-rank test. Multiple linear regression was used to identify predictors of treatment response, including age, sex, baseline platelet count, comorbidities, and blood group. Treatment response was defined as a platelet count ≥50 × 10^9/L or a doubling of baseline platelet count.

Results: Our study included 56 adult patients with primary ITP with mean age 37.25± 16.37 years. Male were 21 (37.5%) and female 35 (62.5%).After first-line therapy,median pre-treatment platelet count 40 × 10^9/L (range 8–92) increased to 98 × 10^9/L (range 30–210), p <0 .001. The overall response rate was 82%. After second-line therapy, median pre-treatment platelet count 52.5 × 10^9/L (range 10–85) increased to 94 × 10^9/L (range 45–180), p < .005. The overall response rate was 75%. Afterthird-line therapy, median pre-treatment platelet count 40 × 10^9/L (range 25–55) increased to 101 × 10^9/L (range 70–150), p = 0.12. Response rate was 67%. Regression analysis identified older age (B = 1.92, p = 0.004) and blood group B (B = 10.60, p = 0.01) as predictors of favorable response, whereas higher baseline platelet count (B = –0.77, p = 0.008) and presence of comorbidities (B = –48.930, p =0.01) were associated with poorer outcomes. Sex was not a significant predictor.

Conclusion: This single-center study underscores the stepwise treatment approach for ITP: corticosteroids followed by thrombopoietin receptor agonists and splenectomy. Patient-specific factors such as age, baseline platelet count, comorbidities, and blood group may help tailor individualized treatment strategies. Larger, prospective studies are warranted to validate these findings.

Keywords: Purpura, Thrombocytopenic, Idiopathic; Platelet Count; Corticosteroids/therapeutic use

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Introduction

Immune thrombocytopenia (ITP) is a challenging autoimmune disorder resulting in immune mediated platelet destruction.[1] This leads to isolated thrombocytopenia (platelet count <100 × 10^9/L), heightening the risk of bleeding.[2] ITP can occur at any age and is particularly difficult to treat in adults, where it often becomes a chronic condition, affecting an estimated 3.3 per 100,000 person-years globally, with a higher burden among women and the elderly.[3]

Navigating the treatment landscape for ITP follows a well-established, yet often difficult, path. First line treatment is sually corticosteroids, to quickly improve platelet counts and mitigate bleeding risk.[4] However, many patients either do not respond or relapse on steroid taper creating the need for second-line options. Second line options wary. These include thrombopoietin receptor agonists (TPO-RAs) like eltrombopag, , or to immunosuppressants like azathioprine and rituximab.[5] For those who remain refractory to these medications, splenectomy stands as a potent, albeit declining, third-line option, a trade-off that balances therapeutic benefit against the risks of surgery and long-term infection. [6]

While international guidelines provide a clear roadmap for this journey, real-world clinical practice is often shaped by local terrain—especially in emerging healthcare systems where access to and affordability of treatments like TPO-RAs can be significant hurdles.[7] This creates a critical knowledge gap: how are these treatment pathways truly being navigated outside of the high-income settings where most research originates?[8,9] Compounding this issue is the crucial question of who responds best. Clinical outcomes are not uniform, yet patient-specific predictors like age, baseline platelet

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count, comorbidities, and even blood group remain underexplored in many predictive models.[10]

Therefore, this study sought to map the real-world treatment patterns and their effectiveness among adult primary ITP patients at our single tertiary care center. We aimed to not only document our institutional practices—from first-line steroids to third-line interventions—but also to identify key clinical and demographic predictors that influence therapeutic outcomes. We hypothesized that our patterns would broadly align with established norms but that specific factors like older age and blood group would emerge as positive predictors of response, while comorbidities and higher initial platelet counts would correlate with poorer outcomes.

Methodology

We conducted a retrospective observational study at Combined Military Hospital, Rawalpindi to investigate real-world treatment patterns for adult primary ITP. Our cohort included all eligible patients aged 18 years or older diagnosed with primary ITP between January 2023 and December 2024. In line with established diagnostic guidelines, a diagnosis of primary ITP required the systematic exclusion of secondary causes, including viral infections (HIV, HBV, HCV), concurrent autoimmune diseases, and lymphoproliferative disorders. Bone marrow examinations were performed when clinically necessary to confirm the diagnosis.

Ethics approval was obtained from the Institutional Review Board (IRB no. Ref: IRB-016/AFBMTC/Approval/2024).

Using a standardized data abstraction form, we systematically reviewed patient electronic medical records (EMRs) to extract key information. The data collected included patient demographics (age, sex), clinical characteristics (comorbidities, ABO blood group), and laboratory values, specifically baseline and post-treatment platelet counts. We also meticulously documented the treatment regimens administered, categorizing them into a clear therapeutic hierarchy: first-line (primarily corticosteroids), second-line (e.g., eltrombopag, azathioprine, rituximab), and third-line (primarily splenectomy) to map each patient's treatment journey.

Our primary outcome was the change in platelet count after each line of therapy. To assess this, we compared pre- and post-treatment platelet levels. As the platelet data were not normally distributed, we used the non-parametric Wilcoxon signed-rank test for this comparison. To address our secondary outcome—identifying factors that predict treatment response—we constructed a multiple linear regression model. This model evaluated the influence of several covariates: age, sex, baseline platelet count, comorbidity status, and blood group. We used beta coefficients and their corresponding p-values to interpret the strength and direction of these associations. All statistical analyses were performed using SPSS Version 29.

This study was conducted in full compliance with ethical standards and received approval from the hospital's Institutional Review Board (IRB). Due to the retrospective nature of the analysis and the use of de-identified patient data, the IRB granted a waiver of the requirement for individual informed consent, consistent with local regulations.

Results

Our study included 56 adult patients with primary ITP with mean age 37.25± 16.37 years. Male were 21 (37.5%) and female 35 (62.5%) The treatment approach followed a clear, escalating pathway, starting with widely accepted first-line agents and moving to more specialized therapies as needed.

The cornerstone of initial therapy was corticosteroids, used either alone (57.4%) or in combination (e.g., with azathioprine, 12.8%) in the 47 patients who received first-line treatment (Table I). This initial strategy proved highly effective. We observed a significant rise in median platelet counts, more than doubling from 40 (IQR: 19–54) to 98 (IQR: 50–105) × 10^9/L ($p < .001$, Table 2). An overwhelming majority of patients (91.5%) demonstrated a complete response, with only a small fraction (8.5%) failing to show improvement.

For the 16 patients requiring further intervention, the most common second-line agents were eltrombopag (37.5%) and azathioprine (31.25%). This approach also yielded significant clinical benefits, boosting median platelet counts from 52.5 (IQR: 35.5–84.25) to 94 (IQR: 42–154.75) × 10^9/L ($p < .005$, Table II). The response rate remained high at 81.3%. For the three patients with

refractory disease requiring third-line therapy, two underwent a splenectomy and one received rituximab. While all three showed an increase in platelets (median 40 to 101 × 10^9/L) (p = 0.10) (Table II).

Table 1. Treatment patterns by line of therapy			
	Drugs	n	%
First Line Treatment (n=47)	Azathioprine	1	2.13
	Eltrombopag	4	8.51
	Steroids + Azathioprine	15	31.86
	Steroids Alone	27	57.40
Second Line Treatment (n=16)	Azathioprine	5	31.25
	Azathioprine + Eltrombopag	2	12.50
	Eltrombopag	6	37.50
	Rituximab	1	6.25

Table II: Evaluation of treatment responses		
Before	After	p-value
Median (IQR)	Median (IQR)	
Platelet response to first-line treatment (n = 47)		
40 (19 – 54)	98 (50 – 105)	0.001
Platelet response to second-line treatment (n = 16)		
52.5 (35.5 – 84.25)	94 (42 – 154.75)	0.001
Platelet response to third-line treatment (n = 3)		
40 (28 – 48)	101 (98 – 128)	0.10
Note : Wilcoxon signed rank test was applied, p<0.05 is significant		

Table III: Predictors of treatment response (multivariate regression analysis)				
Model			95% C.I. for EXP(B)	
	B	P-Value	Lower	Upper
Age	1.922	0.004	.643	3.201
PLT baseline	-0.771	.008	-1.333	-.209
Sex	-6.417	.681	-37.685	24.851
Comorbidities	-48.930	.018	-88.952	-8.907
Blood group	10.608	.018	1.673	19.542
Constant	50.463	.021	-15.208	116.134
R Square =0.306				

	Rituximab + Eltrombopag	1	6.25
	Splenectomy	1	6.25
Third Line Treatment (n=3)	Rituximab	1	33.34
	Splenectomy	2	66.66

A multiple linear regression analysis was performed to identify variables associated with treatment response. (Table III) Several factors demonstrated statistically significant associations. Older age was positively correlated with treatment response (p-value = 0.004). In contrast, a higher baseline platelet 10^9/L (B = -0.771, p-value = 0.008) and the presence of comorbidities (B = -48.93, p-value = 0.01) were associated with lower response rates. Blood group B also emerged as a significant positive predictor (B = 10.60, p-value = 0.01), indicating a potential biological influence on therapeutic outcomes. No statistically significant relationship was observed between treatment response and sex (p-value = 0.68).

Discussion

This investigation provides a comprehensive overview of ITP management in a tertiary care setting, confirming the persistence of established treatment algorithms while also identifying patient-specific factors with potential to refine therapeutic decision-making. Among our studied cohort, a significant proportion of patients required escalation to second line (28.6%) and third line therapy (5.4%). Data from a multicenter Malaysian cohort describes similar escalation patterns in a LMIC.[7] Patterns of treatment differ across different health systems with western data demonstrating earlier adoption of second line therapy and improved platelet counts and bleeding outcomes.[11] This likely reflects access and availability constraints as physicians in our setting may extend steroid courses and prefer selective and late escalation in patients due to limited availability of TPO-Ras and biologics.

In keeping with current guidelines [12], the predominant first line therapy among our population was steroid monotherapy (57.4%), while a significant proportion of patients received steroids in combination with azathioprine (31.86%). Evidence for the use of azathioprine as upfront therapy for adult ITP is limited; subgroup studies on ANA positive patients show improved complete response rates with azathioprine combination therapy compared to steroids alone (60.0% versus 22.2% P = 0.038).[13] Azathioprine was used as a steroid sparing alternative (most frequently in steroid dependent cases), prioritizing low-cost immunosuppression in real world practice when access to TPO-RAs, rituximab, or splenectomy is limited, reflecting an access based approach to reduce steroid exposure while providing feasible immunomodulatory effect in resource constrained settings. [14]

Following first line therapy, our study demonstrated a significant increase in platelet counts (P <0.001). These results reinforce the established role of corticosteroids in achieving rapid improvement in adult primary ITP.

Results from our studies align with expert evidence syntheses, highlighting that upfront steroid therapy results in rapid initial responses. However, sustained remissions are less common. This emphasizes on the need for steroid sparing treatment in relapsed or refractory disease .[6,15]

Second line therapy was associated with a significant increase in platelet counts in our cohort. For patients requiring second line therapy, eltrombopag and azathioprine were the most commonly used agents, while the use of combination therapy with azathioprine and eltrombopag, and rituximab remained limited. Second line treatment in high income settings centers around rituximab (73.3%) and TPO-RAs (5.8%). Results from Podstawka et al. show that TPO-RA is associated with longer median RFS (6.3 vs 3.8 years, P = 0.06) compared with rituximab, and similar rates of ITP-related hospitalizations, major bleeding, and thromboembolism.[16] Similarly, data from Lal et al. shows eltrombopag yielded the highest proportion of patients with completely treatment free periods of ≥180 days.[17] In our setting the preference for eltrombopag and azathioprine is driven by reduction in access and affordability to rituximab, infection concerns, and the need for careful monitoring.

Among patients who required third line therapy there was no statistically significant increase in platelet counts. This is likely attributable to the small sample size rather than lack of effect. Third line interventions, especially splenectomy, are used less frequently as medical therapies have evolved over time, leading to decreased surgical acceptability, due to increased infection risks, the need for vaccinations and follow-up, as well as local resource constraints.

In our multivariate model older age and blood group B were positive predictors of response to therapy. Large retrospective analysis by lam-arunthai et al. showed that age >26 years was associated with an increased response to first line treatment (OR 5.09, 95% CI 1.50-17.28, p = 0.009).[18] Our results are consistent with these findings, suggesting that older patients carry a higher bleeding risk and are therefore, likely to be treated earlier, with prolonged treatment, monitored more closely, and managed with stricter platelet thresholds. Furthermore, older age has been shown to increase the risk of steroid refractoriness in patients with ITP [19], suggesting that the relationship between age and treatment response is contextual and affected by population type and resistance patterns.

There is lack of strong evidence supporting the association of ABO blood group with response to therapy among ITP patients. A retrospective analysis by Laharwal et al. does not report any significant relationship between ABO blood group and response to therapy.[20] Our results demonstrate an exploratory association in a single centre study. Further studies are required to investigate the causal relationship between blood group and treatment response.

Higher comorbidity burden and a low baseline platelet count were found to be negative predictors of response to treatment in our sample population. Bensouda et al. found a higher baseline platelet count to be the sole predictor of chronicity in patients with ITP.[21] These findings reflect a more chronic, ITP phenotype where platelet counts are moderately low but less likely to show steroid-related increase. A higher comorbidity burden as shown by Mannering et al. results in reduced observed treatment response. Such patients require individualized regimens with dose limitations, higher risk of adverse effects, drug-drug interactions and competing causes of thrombocytopenia or bleeding. [22]

Several limitations of this study should be recognized. The retrospective design is prone to missing or incomplete data and the relatively small number of patients receiving second- and third-line therapies restricts statistical power.

Future multi-center prospective studies with standardized response timepoints, bleeding outcomes, durability (relapse-free survival), and adverse events are needed to validate these real-world treatment patterns and predictors.

Conclusion

This study reaffirms the established treatment sequence for primary ITP, with corticosteroids as the first-line standard followed by thrombopoietin receptor agonists and immunosuppressants. Beyond confirming this therapeutic ladder, we demonstrate that treatment response is strongly influenced by patient-specific factors, including age, comorbidities, baseline platelet count, and blood group.

These findings highlight the need to move from a uniform treatment approach towards a predictive, personalized approach, with particular relevance in resource-limited settings where treatment decisions must be optimized based on accessibility and affordability. Larger prospective multicenter studies are required to validate these predictors and translate them into evidence-based algorithms for individualized patient care.

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