

Clinical Spectrum and Early Treatment Response in Pediatric Immune Thrombocytopenia: A Prospective Observational Study

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ABSTRACT

Background: Immune thrombocytopenia (ITP) is the most common acquired autoimmune bleeding disorder in children and is characterized by isolated thrombocytopenia resulting from immune-mediated platelet destruction and impaired platelet production. Although most pediatric patients experience spontaneous recovery or respond favorably to first-line therapy, considerable heterogeneity exists in clinical presentation and early treatment outcomes. This study aimed to evaluate the clinical spectrum of pediatric ITP and identify factors associated with early treatment response. **Methods:** This prospective observational study was conducted in the Departments of Pediatrics at Mymensingh Medical College Hospital (MMCH) and Community Based Medical College Hospital Bangladesh (CBMCHB), from June 2025 to July 2026. A total of 125 consecutive children (<18 years) with newly diagnosed primary ITP were enrolled. Demographic characteristics, clinical manifestations, laboratory parameters, treatment modalities, and early treatment outcomes were recorded using a structured data collection form. Associations between treatment modality and treatment response were assessed using the Fisher–Freeman–Halton exact test, while independent predictors of complete early response were identified using multivariable binary logistic regression. Statistical analyses were performed using IBM SPSS Statistics version 26.0, with a two-sided p-value <0.05 considered statistically significant. **Results:** The mean age of the participants was 5.8 ± 3.9 years, and 58.4% were male. Petechiae (87.2%) and purpura (64.8%) were the most frequent presenting manifestations, whereas intracranial hemorrhage occurred in only

one patient (0.8%). The mean baseline platelet count was $13.4 \pm 8.2 \times 10^9/L$. Corticosteroids were the most commonly administered first-line therapy (57.6%), followed by IVIG (19.2%). Early treatment response differed significantly across treatment modalities ($p=0.031$), with complete response rates of 70.8% for corticosteroids, 87.5% for IVIG, and 100.0% for combined corticosteroid plus IVIG therapy. Multivariable logistic regression demonstrated that age <5 years (adjusted odds ratio [AOR] 1.82, 95% confidence interval [CI] 1.04–3.20; $p=0.036$), baseline platelet count $\geq 10 \times 10^9/L$ (AOR 2.74, 95% CI 1.39–5.41; $p=0.004$), and initial IVIG therapy (AOR 3.18, 95% CI 1.28–7.89; $p=0.012$) were independent predictors of complete early treatment response. **Conclusions:** Pediatric ITP in this cohort predominantly presented with mild-to-moderate mucocutaneous bleeding and demonstrated favorable early treatment outcomes. Corticosteroids remained the most frequently used first-line therapy, whereas IVIG achieved superior early response rates. Younger age, higher baseline platelet count, and initial IVIG therapy independently predicted complete early treatment response. These findings provide clinically relevant evidence for individualized risk stratification and therapeutic decision-making in children with newly diagnosed ITP and may contribute to optimizing pediatric hematology practice in resource-limited settings.

Keywords: Immune thrombocytopenia, Children, Pediatric ITP, Autoimmune thrombocytopenia, Intravenous immunoglobulin, Early treatment response.

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INTRODUCTION

Immune thrombocytopenia (ITP) is an acquired autoimmune hematological disorder characterized by isolated thrombocytopenia (platelet count $<100 \times 10^9/L$) resulting from immune-mediated platelet destruction and impaired platelet production. In children, primary ITP is the most common cause of acquired thrombocytopenia and typically presents with sudden onset of petechiae, purpura, ecchymosis, or mucosal bleeding in otherwise healthy individuals. Although the disease is generally self-limiting, a subset of children develops persistent or chronic thrombocytopenia requiring prolonged monitoring and therapeutic intervention ^[1]. The annual incidence of pediatric ITP is estimated to range between 2 and 6 cases per 100,000 children, with the highest incidence occurring among those younger than five years of age. Viral infections frequently

precede disease onset, supporting the concept that immune dysregulation triggered by infectious agents plays a pivotal role in disease pathogenesis. Autoantibodies directed against platelet membrane glycoproteins accelerate platelet clearance through the reticuloendothelial system while simultaneously impairing megakaryocyte maturation and platelet production within the bone marrow ^[2]. Clinical manifestations of pediatric ITP are highly heterogeneous, ranging from asymptomatic thrombocytopenia to extensive mucocutaneous bleeding. Petechiae, purpura, epistaxis, gingival bleeding, and easy bruising represent the most common presenting features, whereas life-threatening hemorrhage, including intracranial bleeding, remains rare, occurring in less than 1% of affected children. Consequently, disease severity is determined primarily by the extent of

bleeding rather than the absolute platelet count, and contemporary management strategies emphasize individualized risk assessment instead of laboratory values alone ^[3]. Over the past decade, the classification of ITP has evolved considerably. Current international consensus guidelines classify the disease into newly diagnosed (<3 months), persistent (3–12 months), and chronic (>12 months) categories, replacing the traditional acute and chronic terminology. This classification better reflects disease evolution, facilitates standardized reporting, and supports evidence-based therapeutic decision-making across pediatric populations ^[4]. Current international recommendations advocate observation alone for children with newly diagnosed ITP who exhibit only minor or no bleeding, irrespective of platelet count, thereby avoiding unnecessary exposure to treatment-related adverse

effects. Conversely, corticosteroids, intravenous immunoglobulin (IVIG), and anti-D immunoglobulin (where appropriate) remain the principal first-line therapies for children presenting with clinically significant mucosal bleeding or impaired health-related quality of life. Recent evidence continues to support a conservative management approach in carefully selected patients while emphasizing prompt intervention in those with clinically important hemorrhagic manifestations^[5]. Despite generally favorable outcomes, treatment response among children with ITP varies substantially. Several clinical and laboratory factors—including younger age, preceding viral infection, abrupt disease onset, severity of thrombocytopenia, bleeding phenotype, and initial treatment modality—have been investigated as potential predictors of early platelet recovery. However, published findings remain inconsistent across different populations, and no universally accepted prognostic model accurately predicts early therapeutic response in pediatric ITP^[6]. The increasing availability of prospective observational studies has improved understanding of the natural history of childhood ITP, yet significant knowledge gaps remain regarding the relationship between presenting clinical characteristics and early treatment outcomes, particularly in low- and middle-income countries. Regional differences in infectious exposures, healthcare access, treatment preferences, and follow-up practices may influence disease presentation and therapeutic response, highlighting the importance of generating locally relevant clinical evidence^[7]. Recent updates to international guidelines continue to reinforce patient-centered management while acknowledging that high-quality pediatric evidence remains limited for several therapeutic decisions. Emerging research has focused on identifying clinical predictors that may distinguish children likely to experience spontaneous remission from those requiring pharmacological intervention or prolonged follow-up. Improved risk stratification could minimize overtreatment while ensuring timely management of patients at increased risk of bleeding complications or persistent disease^[8]. Prospective evaluation of the clinical spectrum of pediatric ITP is particularly valuable because it provides systematic documentation of demographic characteristics, bleeding patterns, laboratory findings, antecedent infections, treatment choices, and short-term therapeutic outcomes under routine clinical practice. Such evidence contributes to optimization of management protocols and enhances

understanding of disease behavior across different healthcare settings^[9]. Furthermore, early treatment response has emerged as an important clinical endpoint because rapid platelet recovery is associated with reduced bleeding risk, shorter hospital stay, decreased healthcare utilization, and improved quality of life for both patients and caregivers. Identification of factors associated with favorable early response may facilitate individualized treatment selection and resource allocation in pediatric hematology practice^[10]. Although several studies have examined predictors of chronicity, comparatively fewer prospective investigations have focused specifically on determinants of early treatment response during the initial phase of disease. This limitation is particularly evident in South Asian populations, where epidemiological and clinical data remain scarce despite substantial disease burden. Additional prospective evidence is therefore required to guide context-specific management strategies and improve clinical outcomes^[11]. Against this background, the present prospective observational study was conducted to evaluate the clinical spectrum of pediatric immune thrombocytopenia and to assess early treatment response among affected children. The study also aimed to identify demographic, clinical, and laboratory factors associated with early therapeutic outcomes, thereby contributing contemporary evidence to support evidence-based management of pediatric ITP in routine clinical practice^[12].

METHODS & MATERIALS

This prospective observational study was conducted in the Departments of Pediatrics at Mymensingh Medical College Hospital (MMCH) and Community Based Medical College Hospital Bangladesh (CBMCHB), over a one-year period from June 2025 to July 2026. The study included 125 consecutive pediatric patients (<18 years) with newly diagnosed primary immune thrombocytopenia (ITP), defined as an isolated platelet count of $<100 \times 10^9/L$ according to internationally accepted diagnostic criteria. Patients with secondary ITP, congenital or inherited thrombocytopenia, previous ITP treatment, incomplete clinical records, or loss to follow-up before assessment of early treatment response were excluded. Written informed consent was obtained from the parents or legal guardians of all participants before enrollment. Data were collected prospectively using a structured data collection form. Information on sociodemographic characteristics, clinical presentation, history of recent

viral infection, duration of symptoms, and bleeding manifestations was recorded. Baseline laboratory investigations included complete blood count, platelet count, hemoglobin level, total white blood cell count, and mean platelet volume (MPV). Patients received appropriate management according to institutional protocols and current international guidelines, including observation, corticosteroids, intravenous immunoglobulin (IVIG), combined corticosteroid and IVIG therapy, or platelet transfusion when clinically indicated. Early treatment response was classified as complete response, partial response, or no response based on standardized platelet response criteria. Data were entered into Microsoft Excel and analyzed using IBM SPSS Statistics version 26.0 (IBM Corp., Armonk, NY, USA). Continuous variables were expressed as mean $\pm$ standard deviation (SD), while categorical variables were presented as frequencies and percentages. The association between treatment modality and early treatment response was evaluated using the Fisher–Freeman–Halton exact test. Factors associated with complete early treatment response were identified using binary logistic regression analysis, and results were reported as adjusted odds ratios (AORs) with 95% confidence intervals (95% CIs). A two-tailed p -value <0.05 was considered statistically significant. Written informed consent was obtained from the parents or legal guardians of all participants, and assent was obtained from older children whenever appropriate. Participant confidentiality was maintained throughout the study by anonymizing all collected data. The study was conducted in accordance with the ethical principles of the Declaration of Helsinki and subsequent amendments.

RESULTS

A total of 125 pediatric patients diagnosed with immune thrombocytopenia (ITP) were included in the study. The mean age of the participants was 5.8 ± 3.9 years. The largest proportion of patients belonged to the 1–5 years age group (41.6%), followed by those aged 6–10 years (27.2%), 11–18 years (16.8%), and infants younger than one year (14.4%). Male patients constituted 58.4% of the study population, while females accounted for 41.6%, resulting in a male-to-female ratio of approximately 1.4:1. Slightly more than half of the participants (54.4%) resided in urban areas, whereas 45.6% were from rural settings (*Table 1*).

Table I
Sociodemographic Characteristics of the Study Participants.

Variable	Frequency (n)	Percentage (%)
Age group (years)		
<1	18	14.4
1–5	52	41.6
6–10	34	27.2
11–18	21	16.8
Mean age (years)	5.8 ± 3.9	
Sex		
Male	73	58.4
Female	52	41.6
Residence		
Urban	68	54.4
Rural	57	45.6

The most common clinical manifestation at presentation was petechiae, observed in 87.2% of the patients, followed by purpura (64.8%) and ecchymosis (35.2%). Mucosal bleeding manifestations included epistaxis in 23.2% and gum bleeding in 16.0% of cases. Less frequent but clinically significant bleeding manifestations were hematuria (4.8%), gastrointestinal bleeding (3.2%), and intracranial hemorrhage, which occurred in only one patient (0.8%). A recent history of viral infection was documented in 37.6% of patients, while 25.6% presented with fever. Most patients (66.4%) sought medical attention within seven days of symptom onset (*Table II*).

Table II
Clinical Characteristics of the Patients.

Variable	Frequency (n)	Percentage (%)
Petechiae	109	87.2
Purpura	81	64.8
Ecchymosis	44	35.2
Epistaxis	29	23.2
Gum bleeding	20	16.0
Hematuria	6	4.8
Gastrointestinal bleeding	4	3.2
Intracranial hemorrhage	1	0.8
Recent viral infection	47	37.6
Fever	32	25.6
Symptom duration <7 days	83	66.4

Baseline laboratory investigations revealed a mean platelet count of $13.4 \pm 8.2 \times 10^9/L$, reflecting severe thrombocytopenia at diagnosis. The mean hemoglobin concentration was $11.4 \pm 1.5 \text{ g/dL}$, while the mean white blood cell count was $8.7 \pm 2.8 \times 10^9/L$. The average mean platelet volume (MPV) was $10.5 \pm 1.3 \text{ fL}$, indicating relatively large circulating platelets, a finding commonly observed in pediatric immune thrombocytopenia (*Table III*).

Table III
Baseline Laboratory Profile.

Variable	Mean ± SD
Platelet count ($\times 10^9/L$)	13.4 ± 8.2
Hemoglobin (g/dL)	11.4 ± 1.5
WBC count ($\times 10^9/L$)	8.7 ± 2.8
MPV (fL)	10.5 ± 1.3

Regarding initial management, corticosteroid therapy was the most frequently administered treatment, accounting for 57.6% of patients. Intravenous immunoglobulin (IVIG) was used in 19.2%, whereas 14.4% of children were managed conservatively with observation alone. Combination therapy with corticosteroids and IVIG was administered to 6.4% of patients, and platelet transfusion was required in only 2.4%, primarily in severe bleeding situations (*Table IV*).

Table IV
Initial Treatment Modalities ($n = 125$).

Treatment	Frequency (n)	Percentage (%)
Observation	18	14.4
Corticosteroids	72	57.6
IVIG	24	19.2
Steroid + IVIG	8	6.4
Platelet transfusion	3	2.4

Early treatment response differed significantly according to the initial therapeutic modality ($p = 0.031$). The highest complete response rate was achieved among patients receiving combined corticosteroid and IVIG therapy (100.0%), followed by those treated with IVIG alone (87.5%) and corticosteroids alone (70.8%). Patients managed by observation demonstrated a complete response in 55.6% of cases, whereas those requiring platelet transfusion had the lowest complete response rate (33.3%). These findings indicate that treatment modality was significantly associated with early therapeutic outcomes (Table V).

Table V
Early Treatment Response According to Initial Treatment Modality.

Treatment	Complete Response n (%)	Partial Response n (%)	No Response n (%)	p-value
Observation (n=18)	10 (55.6)	6 (33.3)	2 (11.1)	0.031
Corticosteroids (n=72)	51 (70.8)	17 (23.6)	4 (5.6)	
IVIG (n=24)	21 (87.5)	3 (12.5)	0 (0.0)	
Corticosteroids + IVIG (n=8)	8 (100.0)	0 (0.0)	0 (0.0)	
Platelet transfusion (n=3)	1 (33.3)	1 (33.3)	1 (33.3)	

P-value was calculated using Fisher–Freeman–Halton Exact Test because several cells had expected frequencies less than five.

Multivariable binary logistic regression identified several independent predictors of complete early treatment response. Children younger than 5 years were significantly more likely to achieve a complete response than older children (Adjusted OR = 1.82, 95% CI: 1.04–3.20; $p = 0.036$). Patients with an initial platelet count of $\geq 10 \times 10^9/L$ had nearly a threefold higher likelihood of complete response (Adjusted OR = 2.74, 95% CI: 1.39–5.41; $p = 0.004$). Similarly, initial treatment with IVIG was independently associated with improved treatment response (Adjusted OR = 3.18, 95% CI: 1.28–7.89; $p = 0.012$). In contrast, male sex (Adjusted OR = 1.21, $p = 0.542$) and a recent viral infection (Adjusted OR = 0.89, $p = 0.734$) were not significantly associated with complete early treatment response (Table VI).

Table VI
Factors Associated with Complete Early Treatment Response.

Variable	Adjusted OR	95% CI	p-value
Age <5 years	1.82	1.04–3.20	0.036
Male sex	1.21	0.65–2.27	0.542
Platelet count $\geq 10 \times 10^9/L$	2.74	1.39–5.41	0.004
Recent viral infection	0.89	0.46–1.72	0.734
Initial IVIG therapy	3.18	1.28–7.89	0.012

Binary logistic regression was performed using complete early treatment response (yes/no) as the dependent variable. Partial response and no response were combined into the "incomplete response" category.

DISCUSSION

The present prospective observational study provides contemporary evidence regarding the demographic profile, clinical manifestations, and baseline laboratory characteristics of children with newly diagnosed primary immune thrombocytopenia (ITP) in two tertiary care hospitals in Bangladesh. Our findings demonstrate that pediatric ITP predominantly affected younger children, with more than half of the patients being younger than five years of age and a mean age of 5.8 ± 3.9 years. This age distribution is consistent with previous studies reporting that childhood ITP most frequently occurs during the preschool years, reflecting the age at which immune-mediated responses following common viral infections are most prevalent [13]. A slight male predominance (58.4%) was observed in the present study, which is comparable to several recent pediatric ITP cohorts reporting a modest excess of male patients during childhood, although sex differences generally diminish with increasing age [14]. The predominance of urban residents (54.4%) in our cohort may reflect differences in healthcare accessibility and referral patterns rather than a true epidemiological variation, as previous investigations have not consistently

demonstrated an association between residence and the incidence of pediatric ITP [15]. Petechiae (87.2%) represented the most frequent presenting manifestation, followed by purpura (64.8%) and ecchymosis (35.2%). These findings are in agreement with contemporary literature describing cutaneous bleeding manifestations as the hallmark clinical features of newly diagnosed pediatric ITP [16]. Mucosal bleeding, including epistaxis (23.2%) and gum bleeding (16.0%), occurred less frequently but remained clinically important because these manifestations often influence the decision to initiate pharmacological treatment rather than observation alone [17]. Major bleeding complications were uncommon in the present study. Gastrointestinal bleeding occurred in only 3.2% of patients, hematuria in 4.8%, and intracranial hemorrhage was documented in a single child (0.8%). These findings support existing evidence indicating that severe or life-threatening bleeding is rare in childhood ITP despite profoundly reduced platelet counts [18]. The low incidence of intracranial hemorrhage observed in our cohort is particularly reassuring and is consistent with large international studies demonstrating that this complication occurs in fewer than 1% of

pediatric patients [19]. A history of recent viral infection was identified in 37.6% of participants, while approximately one-quarter presented with fever. Viral illnesses are widely recognized as important triggers for immune dysregulation leading to autoantibody-mediated platelet destruction, particularly in younger children [20]. Furthermore, nearly two-thirds of our patients presented within seven days of symptom onset, indicating the acute presentation that characterizes most cases of childhood ITP and facilitating timely diagnosis and initiation of appropriate management [21]. The baseline laboratory profile of our study population demonstrated severe thrombocytopenia, with a mean platelet count of $13.4 \pm 8.2 \times 10^9/L$, while mean hemoglobin concentration (11.4 ± 1.5 g/dL) and total white blood cell count ($8.7 \pm 2.8 \times 10^9/L$) remained largely within expected ranges. These findings are characteristic of primary ITP, in which isolated thrombocytopenia is the principal hematological abnormality without significant alterations in other blood cell lineages [22]. The mean platelet volume (MPV) of 10.5 ± 1.3 fL observed in our patients is also consistent with previous studies demonstrating relatively enhanced platelet size in ITP due to enhanced

production of young, metabolically active platelets by the bone marrow in response to accelerated peripheral destruction [23]. Overall, the demographic characteristics, bleeding profile, and laboratory findings observed in the present study closely resemble those reported in contemporary international pediatric ITP cohorts. These results suggest that the clinical spectrum of childhood ITP in Bangladesh is comparable to that reported in other regions and further support current concepts regarding the epidemiology and initial presentation of this autoimmune disorder [24]. The present study demonstrated considerable variation in the initial treatment approaches for children with newly diagnosed immune thrombocytopenia, reflecting the individualized management strategies recommended by current international guidelines [2,3]. Patients with mild bleeding manifestations were more likely to undergo observation, whereas corticosteroids and intravenous immunoglobulin (IVIG) were administered to children with clinically significant bleeding or those requiring rapid platelet recovery [1,2]. Corticosteroids were the most frequently prescribed first-line treatment in the present study, accounting for 57.6% of all patients, indicating that they remain the preferred initial therapeutic option in routine clinical practice because of their effectiveness, availability, and relatively low cost [1,17]. IVIG was used in 19.2% of patients and achieved a higher complete response rate than corticosteroid therapy alone, which is consistent with previous evidence demonstrating its ability to induce rapid platelet recovery in children with acute ITP [2,14]. Combined corticosteroid and IVIG therapy resulted in a 100% complete response in our cohort; however, this finding should be interpreted cautiously because only eight patients received combination treatment, limiting the precision and generalizability of this estimate [2,20]. A statistically significant association was observed between the initial treatment modality and early treatment response ($p=0.031$). This finding suggests that treatment selection has an important influence on short-term clinical outcomes and supports current recommendations that therapeutic decisions should primarily be based on bleeding severity and overall clinical status rather than platelet count alone [1,2,4]. Although platelet transfusion was administered to only a few patients with severe bleeding, its relatively poor response rate was expected because platelet transfusion provides only transient platelet support and does not modify the underlying autoimmune process responsible for platelet destruction [2,17]. Binary logistic regression identified several independent predictors of complete early treatment response. Children younger than five years had significantly greater odds of achieving complete response than older children, suggesting that younger

age may be associated with a more favorable disease course and higher rates of spontaneous immune recovery [14,21]. Likewise, an initial platelet count of $\geq 10 \times 10^9/L$ independently predicted successful early treatment response, indicating that patients with relatively higher baseline platelet counts may experience less severe immune-mediated platelet destruction and better hematological recovery following first-line therapy [22,24]. Initial IVIG therapy remained an independent predictor of complete early treatment response after adjustment for potential confounding variables. This observation is biologically plausible because IVIG rapidly blocks Fc receptor-mediated platelet clearance and modulates autoimmune activity, resulting in prompt elevation of platelet counts during the acute phase of disease [2,17]. In contrast, neither male sex nor a recent viral infection demonstrated an independent association with treatment outcome, suggesting that these variables have limited prognostic value after accounting for other clinical and laboratory factors [14,24]. The prospective design represents a major strength of the present study, as it enabled systematic collection of clinical and laboratory data while minimizing recall bias. Furthermore, the inclusion of consecutive patients from two tertiary care hospitals enhances the representativeness of the study population and reflects routine clinical practice in Bangladesh [15,20]. The use of multivariable logistic regression further strengthened the analysis by identifying independent predictors of early treatment response while controlling for potential confounding factors [24]. Several limitations should also be considered when interpreting the findings. First, the relatively modest sample size may have reduced the statistical power for subgroup analyses, particularly for patients receiving combination therapy or platelet transfusion. Second, treatment allocation was based on physician judgment rather than randomization, introducing the possibility of selection bias. Finally, the study evaluated only early treatment response and therefore could not assess long-term remission, chronic ITP, relapse, or late treatment-related complications [11,21]. In conclusion, the present study demonstrates that pediatric immune thrombocytopenia generally has a favorable early prognosis when managed according to contemporary treatment recommendations. Corticosteroids remained the most frequently used first-line therapy, whereas IVIG achieved the highest rate of complete early response. Younger age, a baseline platelet count of $\geq 10 \times 10^9/L$, and initial IVIG therapy were identified as significant predictors of successful early treatment. These findings may assist clinicians in optimizing individualized treatment strategies and improving early outcomes among children

with newly diagnosed immune thrombocytopenia [1,2,3].

CONCLUSION

This prospective observational study provides valuable insights into the clinical spectrum and early treatment response of pediatric immune thrombocytopenia (ITP) in a tertiary care setting in Bangladesh. Most patients presented during early childhood with typical mucocutaneous bleeding manifestations and severe thrombocytopenia, while life-threatening bleeding events were uncommon. Corticosteroids remained the most frequently used first-line treatment, whereas intravenous immunoglobulin (IVIG) was associated with the highest rate of complete early response. Younger age, a baseline platelet count of $\geq 10 \times 10^9/L$, and initial IVIG therapy were identified as significant predictors of favorable early treatment outcomes. These findings support current evidence-based management strategies and may assist clinicians in optimizing individualized treatment decisions and improving the early prognosis of children with newly diagnosed ITP.

RECOMMENDATIONS

Based on the findings of this study, children with suspected immune thrombocytopenia should undergo prompt clinical evaluation to facilitate early diagnosis and appropriate risk stratification. Treatment decisions should be individualized according to bleeding severity, clinical condition, and patient-specific factors rather than platelet count alone. Intravenous immunoglobulin may be considered for patients requiring rapid platelet recovery, while careful observation remains appropriate for selected children with mild bleeding manifestations. Future multicenter prospective studies with larger sample sizes and extended follow-up are recommended to evaluate long-term outcomes, relapse rates, chronic disease progression, and the effectiveness of emerging therapeutic strategies in the pediatric population.

LIMITATIONS

This study has several limitations. First, it was conducted in only two tertiary care hospitals, which may limit the generalizability of the findings to other healthcare settings. Second, the relatively modest sample size reduced the statistical power for subgroup analyses, particularly for less frequently used treatment modalities. Third, treatment allocation was based on routine clinical judgment rather than randomization, introducing the possibility of selection bias and residual confounding. Finally, the study

evaluated only early treatment response and did not assess long-term remission, relapse, chronic immune thrombocytopenia, treatment-related adverse events, or health-related quality of life.

AI DECLARATION

Artificial intelligence tools contributed marginally to manuscript preparation and development. All core scientific content was human-verified.

REFERENCES

- Neunert C, Terrell DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. *Blood Adv*. 2019;3(23):3829-3866. DOI: <https://doi.org/10.1182/bloodadvances.2019001380>.
- Provan D, Arnold DM, Bussel JB, et al. Updated international consensus report on the investigation and management of primary immune thrombocytopenia. *Blood Adv*. 2019;3(22):3780-3817. DOI: <https://doi.org/10.1182/bloodadvances.2019000812>.
- Neunert CE, Arnold DM, Grace RF, et al. The 2022 review of the 2019 American Society of Hematology guidelines on immune thrombocytopenia. *Blood Adv*. 2024;8(13):3578-3582. DOI: <https://doi.org/10.1182/bloodadvances.2023012541>.
- Ishiguro A, Mori M, Higashigawa M. Guidelines for Childhood Immune Thrombocytopenia 2022. *J Jpn Soc Pediatr Hematol Oncol*. 2023;60:228-233. DOI:
- Uchiyama T, Nomura O, Horiuchi S, et al. Clinical Practice Guidelines for Childhood Immune Thrombocytopenia 2022. *J Jpn Soc Pediatr Hematol Oncol*. 2022; 59:50-57.
- Hui D, Barbara A, McGill SC. *Guidelines for Pediatric Immune Thrombocytopenia: Rapid Review*. Ottawa: CADTH; 2022. DOI: <https://doi.org/10.51731/cjht.2022.348>.
- Neunert CE, et al. Review of evidence supporting pediatric ITP management recommendations. *Blood Adv*. 2024; 8:3578-3582. DOI: <https://doi.org/10.1182/bloodadvances.2023012541>.
- Chandra J. Immune thrombocytopenia: American society of hematology guidelines, 2019. *Indian pediatrics*. 2020 Jan 1. DOI: <https://doi.org/10.1007/s13312-020-1966-8>.
- Provan D, Stasi R, Newland AC, Blanchette VS, Bolton-Maggs P, Bussel JB, Chong BH, Cines DB, Gernsheimer TB, Godeau B, Grainger J. International consensus report on the investigation and management of primary immune thrombocytopenia. *Blood*. 2010 Jan 14;115(2):168-86. DOI: <https://doi.org/10.1182/blood-2009-06-225565>.
- Neunert CE. Management of newly diagnosed immune thrombocytopenia: can we change outcomes?. *Hematology* 2014, the American Society of Hematology Education Program Book. 2017 Dec 8;2017(1):400-5. DOI: <https://doi.org/10.1182/asheducation-2017.1.400>.
- Mithoowani S, Arnold DM. First-line therapy for immune thrombocytopenia. *Hämostaseologie*. 2019 Aug;39(03):259-65. DOI: <https://doi.org/10.1055/s-0039-1684031>.
- Kühne T. Diagnosis and management of immune thrombocytopenia in childhood. *Hämostaseologie*. 2017;37(01):36-44. DOI: <https://doi.org/10.5482/hamo-16-06-0017>.
- Ignatova AA, Suntsova EV, Pshonkin AV, et al. Platelet function and bleeding at different phases of childhood immune thrombocytopenia. *Sci Rep*. 2021;11:8976. DOI: <https://doi.org/10.1038/s41598-021-88900-6>.
- Kim TO, Despotovic JM. Pediatric immune thrombocytopenia (ITP) treatment. *Annals of Blood*. 2021 Mar 31;6. DOI: <https://doi.org/10.21037/aob-20-96>.
- Kühne T, Berchtold W, Michaels LA, et al. Newly diagnosed immune thrombocytopenia in children and adults: a comparative prospective registry analysis. *Haematologica*. 2016;101:103-110. DOI: <https://doi.org/10.3324/haematol.2011.050799>.
- Suntsova EV, Demina IM, Ignatova AA, Ershov NM, Trubina NM, Dobrynina J, Serkova IV, Supik ZS, Orekhova EV, Hachatryan LA, Kotskaya NN. Bleeding tendency and platelet function during treatment with romiplostim in children with severe immune thrombocytopenic purpura. *International Journal of Hematology*. 2017 Jun;105(6):841-8. DOI: <https://doi.org/10.1007/s12185-017-2207-3>.
- Almizraq RJ, Branch DR. Efficacy and mechanism of intravenous immunoglobulin treatment for immune thrombocytopenia in adults. *Annals of Blood*. 2021 Mar 31;6. DOI: <https://doi.org/10.21037/aob-20-87>.
- Sirotych E, Chowdhury SR, Modi D, et al. Critical bleeding in adults and children with immune thrombocytopenia: a multicenter cohort study. *Blood Adv*. 2025;9:3238-3248. DOI: <https://doi.org/10.1182/bloodadvances.2024015494>.
- Neunert C, Noroozi N, Norman G, Buchanan GR, Goy J, Nazi I, Kelton JG, Arnold DM. Severe bleeding events in adults and children with primary immune thrombocytopenia: a systematic review. *Journal of Thrombosis and Haemostasis*. 2015 Mar;13(3):457-64. DOI: <https://doi.org/10.1111/jth.12813>.
- Lambert MP, Gernsheimer TB. Clinical updates in immune thrombocytopenia. *Hematology Am Soc Hematol Educ Program*. 2017;2017:568-575. DOI: <https://doi.org/10.1182/blood-2017-03-754119>.
- Ito M, Yagasaki H, Kanezawa K, et al. Incidence and outcomes of refractory immune thrombocytopenic purpura in children: a retrospective study. *Sci Rep*. 2021;11:14263. DOI: <https://doi.org/10.1038/s41598-021-93646-2>.
- Kashiwagi H, Kuwana M, Murata M, Shimada N, Takafuta T, Yamanouchi J, Kato H, Hato T, Tomiyama Y. Reference guide for the diagnosis of adult primary immune thrombocytopenia, 2023 edition. *International Journal of Hematology*. 2024 Jan;119(1):1-3. DOI: <https://doi.org/10.1007/s12185-023-03672-1>.
- van Bladel ER, Laarhoven AG, van der Heijden LB, Heitink-Pollé KM, Porcelijn L, van der Schoot CE, De Haas M, Roest M, Vidarsson G, de Groot PG, Bruin MC. Functional platelet defects in children with severe chronic ITP as tested with 2 novel assays applicable for low platelet counts. *Blood, The Journal of the American Society of Hematology*. 2014 Mar 6;123(10):1556-63. DOI: <https://doi.org/10.1182/blood-2013-08-519686>.
- Zhang Y, Wang J, Li X, et al. Study on the influencing factors of the first-line treatment response to primary immune thrombocytopenia in children. *Front Pediatr*. 2025. DOI: <https://doi.org/10.3389/fped.2025.1679463>.