Original Article

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A single-center experience of Immune Thrombocytopenic Purpura: Disease characteristics, treatment options, response to treatment, and side effects

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Abstract

Immune thrombocytopenic purpura (ITP) is an acquired autoimmune disorder characterized by isolated thrombocytopenia due to platelet destruction and impaired production. While global treatment guidelines exist, data on disease characteristics and treatment outcomes in resource-limited settings remain scarce.

To describe the clinical characteristics, treatment responses, and adverse effects among ITP patients managed at Teaching Hospital Jaffna, Sri Lanka.

A retrospective descriptive cross-sectional study was conducted among 154 patients diagnosed with ITP prior to March 2023, each with a minimum one-year follow-up. Data were extracted from medical records using a structured questionnaire. Definitions for diagnosis and response were based on International Working Group (IWG) criteria. Descriptive analysis was performed using SPSS version 23.

The majority of patients were female (80.5%) and aged 18–60 years. Most (66.9%) were asymptomatic at diagnosis. Platelet counts <30 × 10⁹/L were observed in 42.9%, correlating with higher bleeding risk. First-line treatment with corticosteroids achieved a 76.2% complete response rate; IVIG resulted in 67.9% complete response. Relapse occurred in 54.5% of patients, with 73.2% of steroid-treated patients developing dependency. Splenectomy and eltrombopag, used as second-line treatments, achieved complete responses in 78.2% and 72.7% respectively. Steroid-related side effects were common; IVIG and eltrombopag were well-tolerated.

ITP in this cohort was predominantly seen in adult females, with favorable acute treatment responses but significant relapse and steroid dependency. Second-line therapies were effective but not widely accessible. These findings emphasize the need for individualized management strategies and improved access to advanced therapies in resource-limited settings.

Key Words

Immune Thrombocytopenic Purpura, treatment, side effects

Introduction

Immune thrombocytopenic purpura (ITP) is an acquired autoimmune disorder characterized by isolated thrombocytopenia (platelet count <100 × 10⁹/L), primarily due to autoantibody-mediated destruction of platelets and impaired platelet production (1). It presents with a wide spectrum of clinical manifestations, ranging from incidental findings to severe bleeding complications. First-line treatments such as corticosteroids and intravenous immunoglobulin (IVIG) are generally effective in the acute setting, but treatment responses are variable, and relapses are common. Long-term management can be complicated by treatment resistance, steroid dependency, and adverse effects. In resource-limited settings like Sri Lanka, advanced therapies such as thrombopoietin receptor agonists may not be readily accessible, leading to greater reliance on traditional therapies. Despite these challenges, there is a lack of local data on treatment outcomes, response patterns, and adverse effects. Understanding these aspects is essential for optimizing

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care and developing context-specific management strategies. Therefore, this study was conducted to describe the clinical and demographic characteristics of ITP patients at Teaching Hospital Jaffna, evaluate their responses to various treatment modalities, assess treatment-related adverse effects, and determine relapse rates and long-term outcomes in this resource-constrained setting.

ITP is more common in adults and tends to affect females more frequently than males [1,2]. ITP presents with a spectrum of clinical features, ranging from incidental thrombocytopenia to severe bleeding manifestations, including intracranial and gastrointestinal hemorrhage [2,3].

Diagnosis is largely clinical and based on exclusion of other secondary causes such as infections, autoimmune diseases like systemic lupus erythematosus (SLE), malignancies, and drug-induced thrombocytopenia [1,2]. The International Working Group (IWG) has standardized diagnostic definitions and response criteria for both adult and pediatric populations, which facilitates comparative analysis across studies [2].

First-line treatments include corticosteroids and intravenous immunoglobulin (IVIG), both of which have shown good initial response rates. However, many patients relapse or become steroid-dependent, necessitating second-line interventions [3]. Corticosteroids are effective in achieving initial platelet recovery, but their long-term use is limited by adverse effects such as hyperglycemia, weight gain, and gastrointestinal complications [3,4].

IVIG provides rapid platelet increase, especially in cases of significant bleeding or pre-operative preparation, and is generally well-tolerated, although minor allergic reactions can occur [5,3]. Splenectomy has traditionally been considered the definitive second-line treatment, offering durable responses in many patients [6]. However, the risk of post-splenectomy infections remains a concern [6].

The emergence of thrombopoietin receptor agonists (TPO-RAs), such as eltrombopag, has significantly expanded therapeutic options for chronic ITP. Eltrombopag has demonstrated efficacy in both short- and long-term management, with response rates comparable to splenectomy in certain cohorts [5,4]. Its use, however, is often limited in resource-constrained settings due to cost and availability, especially in regions like Sri Lanka [3].

Azathioprine and other immunosuppressive agents (e.g., danazol and dapsone) have been used as alternative therapies in refractory ITP, though evidence of their efficacy is mixed and response times are typically delayed [7,3]. In a Sri Lankan multi-center study, similar challenges were observed regarding delayed responses and limited treatment access, underlining the importance of context-specific data to guide clinical decision-making [3].

This study builds on previous work by evaluating the local disease characteristics, treatment patterns, and therapeutic outcomes in a single tertiary care center in Sri Lanka, offering insights into the real-world challenges of ITP management in resource-limited settings.

Methods

A retrospective descriptive cross-sectional study was conducted among patients diagnosed with immune thrombocytopenic purpura (ITP) who attended the hematology clinic at Teaching Hospital Jaffna. The study included 154 patients diagnosed with ITP before March 2023, each of whom had a minimum follow-up period of one year at the clinic. Ethical clearance for the study was obtained from the institutional ethical review board.

Data were collected using a pre-designed, structured questionnaire, which was used to extract relevant information from patients' medical records. The variables collected included: Socio-demographic data (age, sex, etc.), past medical history, including comorbidities and autoimmune conditions, details of ITP diagnosis, including platelet counts at diagnosis and clinical presentation, treatment modalities administered (e.g., corticosteroids, intravenous immunoglobulin), response to therapy, including duration and type of response, adverse effects associated with treatment.

Definitions for the diagnosis of ITP and criteria for treatment response were based on the International Working Group (IWG) guidelines, ensuring standardization and comparability with global studies. Additionally, autoimmune conditions associated with ITP, such as antiphospholipid syndrome, were defined according to the revised Sapporo criteria.

Descriptive statistical analysis was performed using SPSS version 23. Measures of central tendency (mean, median) and dispersion (standard deviation) were calculated for continuous variables. Categorical variables were expressed as frequencies and percentages. The analysis focused on patient demographics, clinical features at presentation, baseline platelet counts, and treatment responses to corticosteroids and intravenous immunoglobulin (IVIG).

Results

A total of 154 patients diagnosed with immune thrombocytopenic purpura (ITP) were included in the study. The majority of patients were female (n = 124; 80.5%), with most (74.7%) aged between 18 and 60 years. Only 11% were over the age of 60. Geographically, 80.5% of the cohort was from Jaffna, followed by Kilinochchi (8.4%) and Mannar (6.5%).

In terms of clinical presentation, 66.9% of patients were asymptomatic at diagnosis, with thrombocytopenia identified incidentally. Minor mucosal bleeding, particularly oral bleeding, was reported in 28.6%, while severe bleeding manifestations—including intracranial and gastrointestinal hemorrhage—occurred in six patients (3.9%). Platelet counts at presentation were <30 × 10⁹/L in 42.9% of patients, and this group had a higher frequency of bleeding symptoms (Figure 1).

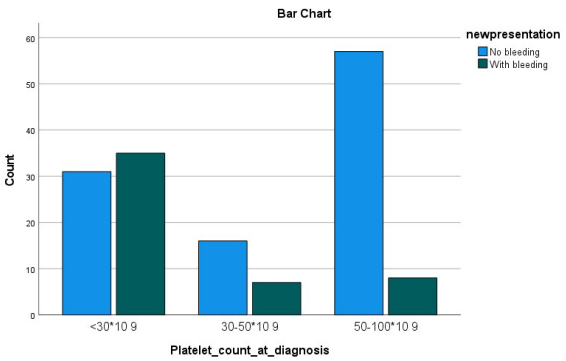

Figure 1: Distribution of bleeding manifestations according to Platelet count at diagnosis in newly presenting ITP patients

Screening for hepatitis B, hepatitis C, and retroviral infections was negative in the majority of patients. Antinuclear antibody (ANA) testing was positive in 25.3%, and 7 patients were diagnosed with systemic lupus erythematosus, indicating secondary ITP.

During acute management, 35.1% of patients were managed conservatively with watchful waiting. Corticosteroids were administered in 42.2% of patients, of whom 76.2% achieved a complete response (Figure 2). IVIG was given to 33.1% of patients, with a 67.9% complete response rate (Figure 3). Sixteen patients received both corticosteroids and IVIG, while 16 also required platelet transfusions.

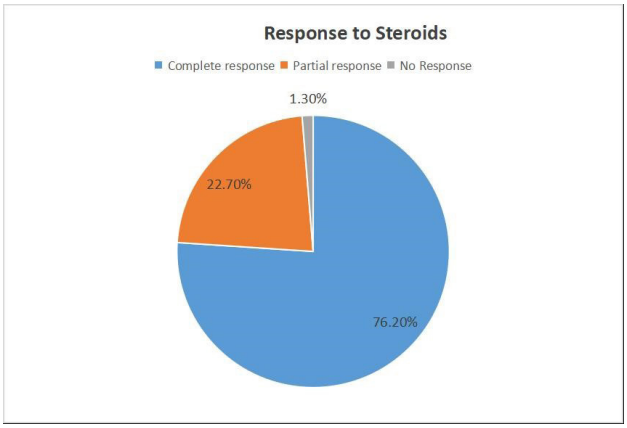

Figure 2: Response to steroid therapy in ITP patients

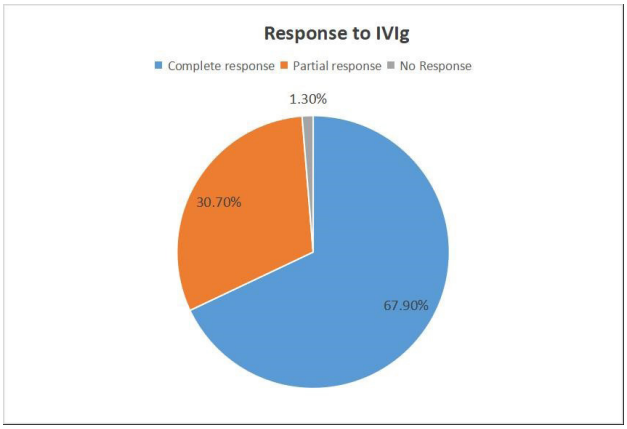

Figure 3: Response to IVIg therapy in ITP patients

In long-term follow-up, 54.5% of patients experienced at least one relapse requiring re-treatment. Among these, 56 patients received additional corticosteroids, and 28 received IVIG. Steroid dependency developed in 73.2% of patients initially treated with corticosteroids. Second-line treatments included splenectomy (performed in 14.9% of patients), which yielded a complete response

in 78.2%, and eltrombopag (used in 7.1% of patients), which achieved a complete response in 72.7%. Other immunosuppressive agents, such as azathioprine, danazol, and dapsone, were used in a small subset of patients but did not show significant efficacy (Figure 4).

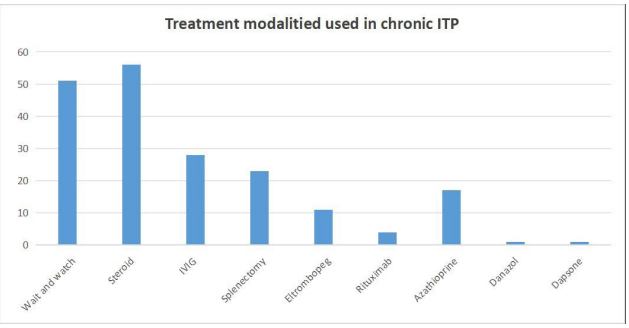

Figure 4: Treatments modalities used in chronic ITP

Adverse effects were predominantly associated with corticosteroid therapy. Hyperglycemia developed in 9 patients, and 16 reported symptoms of peptic ulcer disease. Weight gain was noted in 36 patients, and one developed acne (Figure 5). IVIG was generally well tolerated, with only minor allergic reactions reported in six patients. No adverse effects were observed in patients treated with eltrombopag or rituximab during the study period. One patient developed a post-splenectomy infection. Common comorbidities in the study population included hypertension, diabetes, and hypothyroidism.

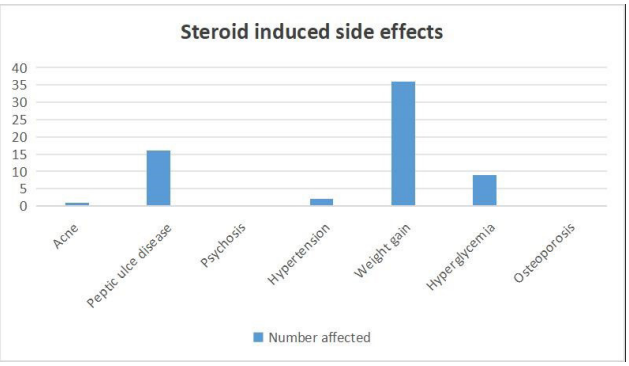

Figure 5: Side effects observed during the treatment with corticosteroid

Discussion

This study provides valuable insights into the demographic characteristics, clinical presentation, treatment responses, and adverse effects among patients with immune thrombocytopenic purpura (ITP) managed at a tertiary care center in Northern Sri Lanka. The findings are largely consistent with global trends, while also highlighting unique challenges faced in resource-limited settings.

The predominance of female patients (80.5%) aligns with the known higher incidence of ITP in women, particularly in the adult population, as previously reported in both local and international studies [8,2,3]. The majority of patients were aged between 18 and 60 years, reflecting the typical age distribution of adult ITP. Notably, over two-thirds of patients were asymptomatic at presentation, and ITP was often diagnosed incidentally—a trend also observed in other cohorts, indicating the need for vigilance in routine blood count evaluations [2].

The severity of bleeding correlated with platelet counts, as expected, with patients presenting with counts $<30 \times 10^9$/L experiencing higher rates of mucosal and severe bleeding. Although severe bleeding was uncommon (3.9%), it emphasizes the importance of early identification and prompt intervention in high-risk patients. The presence of ANA positivity in 25.3% of patients and confirmed systemic lupus erythematosus in 4.5% supports the association of secondary ITP with autoimmune conditions [2,3].

First-line therapies demonstrated favorable initial responses, with corticosteroids achieving a 76.2% complete response rate and IVIG achieving 67.9%. These outcomes are comparable to those reported in multicenter trials and consensus guidelines [1,5,3]. However, a significant proportion of patients (54.5%) experienced relapses, and over 70% of those treated with steroids developed dependency, reflecting the limitations of prolonged corticosteroid use and the need for effective second-line therapies.

Splenectomy, although less commonly performed today due to the availability of newer agents, showed a high response rate (78.2%) in our cohort, reaffirming its role as a potentially curative option in selected patients [6]. Eltrombopag, used in 7.1% of patients, also demonstrated good efficacy (72.7% complete response), consistent with international data on thrombopoietin

receptor agonists [5,4]. However, access to eltrombopag remains limited in Sri Lanka due to cost and availability, highlighting a significant barrier to optimal care.

Azathioprine, danazol, and dapsone were used in small numbers, with limited efficacy. The poor response to azathioprine in this study is consistent with previous reports suggesting that its benefits may be delayed and less predictable [7,3]. Side effects were most commonly associated with steroid use, including hyperglycemia, weight gain, and gastrointestinal symptoms. IVIG was well tolerated, and no significant adverse effects were observed with eltrombopag or rituximab during the study period.

The overall findings emphasize the need for individualized treatment plans that consider disease severity, response patterns, comorbidities, and the availability of therapies. In resource-limited settings, optimizing the use of available first- and second-line treatments while minimizing adverse effects is critical. Furthermore, patient education and long-term follow-up are essential to manage relapses and monitor for treatment-related complications.

Limitations

This study has several limitations that should be considered when interpreting the findings. Firstly, its retrospective design may be subject to information bias due to incomplete or inconsistent documentation in patient records. Secondly, as a single-center study conducted at a tertiary care facility, the findings may not be generalizable to other regions or primary care settings in Sri Lanka. Thirdly, the sample size, while adequate for descriptive analysis, may be insufficient to detect subtle differences in treatment outcomes across subgroups. Additionally, the lack of standardized follow-up intervals and variability in treatment protocols may have influenced the assessment of treatment responses and long-term outcomes. Finally, due to limited availability of newer therapies such as thrombopoietin receptor agonists, the study could not comprehensively evaluate their effectiveness and safety within this population.

Conclusion

First-line treatments such as corticosteroids and IVIG showed favorable initial response rates, but their limitations, particularly in chronic cases, were evident. Second-line therapies, including splenectomy and eltrombopag, demonstrated high efficacy, although access to newer agents remains a challenge in this region.

Further prospective, multicenter studies are warranted to refine treatment strategies and improve outcomes for patients with ITP in similar healthcare settings. Future research should focus on prospective, multicenter studies with larger and more diverse patient populations to validate these findings and improve the generalizability of results across Sri Lanka. Standardizing treatment protocols and follow-up intervals would enhance the accuracy of assessing treatment responses and long-term outcomes. Expanding access to advanced therapies, such as thrombopoietin receptor agonists is essential, and future studies should evaluate their cost-effectiveness and feasibility in resource-limited settings. Additionally, the integration of quality-of-life assessments and patient-reported outcomes into routine care and research would provide a more holistic understanding of the disease burden and treatment impact. Investment in local clinical guidelines tailored to the regional context, supported by robust local data, will be critical for optimizing the management of ITP in Sri Lanka.

Acknowledgments

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