

Health Impact and Burden of Illness in Immune Thrombocytopenia: A Literature Review on Clinical, Humanistic, and Economic Burden

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KEY FINDINGS & CONCLUSIONS

- Patients with primary ITP exhibited a high prevalence of bleeding events and thrombosis, while fatigue emerged as a prominent symptom affecting a considerable proportion of patients.
- ITP imposed a substantial financial burden, with the high costs associated with bleeding events, hospitalizations, ambulatory visits, and prescriptions among other factors.
- Quality of life of patients declined, due to prevalent mental health issues, fatigue, and emotional distress.
- These findings emphasize the need for tailored strategies to enhance disease management and improve the overall patient well-being, especially the most affected groups like the elderly and women.

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INTRODUCTION

- Immune-thrombocytopenia (ITP) is a rare autoimmune-platelet disorder that meets the criteria for an orphan disease, with a prevalence of 1-5 cases per 10,000 population. (1)
- Patients with ITP face a complex set of challenges, and the economic and clinical outcomes can be under-recognized.
- This review aimed to summarize the evidence on clinical, humanistic, and economic burden among ITP patients

METHODS

- Embase and Medline databases, and key conferences of interest were searched from January-2013 to October-2023.
- Studies reporting patients' quality-of-life (QoL), clinical symptoms, healthcare resource utilization (HCRU), and disease management costs were included.

RESULTS

- A total of 67 studies were included (clinical data:65; economic data:25; humanistic data:12).
- Included studies focused on patients with primary ITP, secondary ITP, undefined ITP, and refractory ITP.
- The analysis and discussions mostly centered around studies reporting outcomes for primary ITP or undefined ITP.
- The mean age of patients ranged from 38 to 85.7 years, with a female predominance in most of the studies.

Clinical Burden

- Among ITP patients, the most common symptoms reported were fatigue (43%-94%), bleeding (14%-83%), and treatment-related thrombosis (21.4%-27%).
- Elderly patients (60-89 years) exhibited significantly higher rates of thrombosis (1.7% versus 0.5%, p=0.014) compared to younger patients (18-60 years). (2)
- Women experienced a higher occurrence of overall bleeding and fatigue, with a higher negative impact on their emotional and psychological well-being than that of men (Figure 1).

Table 1. Clinical burden data

Study name	Country	Population type	Patients analyzed (N)	Bleeding events (%)	Thrombosis (%)	Mortality (%)	Fatigue (%)
Cooper, 2023	USA	Primary ITP	18	83	-	-	94
David, 2023	USA	Primary ITP	404	29	24.8	-	-
Goel, 2019	USA	Primary ITP	78,376	17.63	-	1.1	-
Lucchesi, 2023	Italy	Primary ITP	66	-	-	-	64
Palandri, 2020	Italy	Primary ITP	451	-	27	-	-
Palandri, 2021	Italy	Primary ITP	-	-	21.4	-	-
Sokal, 2022	France	Primary ITP	97	54.6	-	3	-
Weber, 2017	France	ITP	36	14	-	5.6	-
Sun, 2023	China	ITP	128	-	-	0.8	-
Zhou, 2013	China	Primary ITP	525	-	-	9.9	-
Cooper, 2021 (a)	Multi-country*	ITP	1507	-	-	-	58
Rovó A, 2022	Multi-country**	Primary ITP	46	59	-	-	43

Abbreviations- USA: United States of America; ITP: Immune Thrombocytopenia
* Canada, China, Colombia, Egypt, France, Germany, India, Italy, Japan, Spain, Turkey, UK and USA
** Switzerland, Austria and Belgium

Economic Burden

- The included studies evaluated HCRU, with a primary focus on hospitalization, physician visits, and intensive-care unit (ICU) stays, highlighted the substantial economic burden of ITP across different regions.
- In the USA, managing ITP involved a considerable healthcare expenditure (\$21,290), with ambulatory encounters (\$12,978) being the major cost driver, followed by hospitalization costs (\$7,802) (Table 2). (3)
- The data indicated that patients with bleeding events incurred significantly higher HCRU than patients without bleeding (hospitalization: 10.2% versus 2.4%; ICU: 0.2% versus 0%, p=0.03). (4)
- Additionally, the cost of treating bleeding events and hospitalization rates were found to increase, as the severity of the events progressed from mild to severe (Table 2). (5)

Table 2. Healthcare expenditure and utilization based economic burden data

Study name	Country	Population type	Patients analyzed	Healthcare expenditure		HCRU (proportion of patients)		
				Cost item; Cost year	Total cost	ICU stay (%)	Physician visits (%)	Hospitalization (%)
David, 2023	USA	Primary ITP	404	-	-	7.7	-	-
Liang, 2021	USA	Primary ITP	135	-	-	-	-	31.1
Lin, 2017	USA	Primary ITP	6,651	Reimbursement cost (per bleeding event); 2015	\$6,022	-	-	-
			2,994	-	-	-	48.7	-
Weycker, 2020	USA	Primary ITP	11,028	ITP-related costs (per patient); 2016	\$21,290	-	-	-
				Cost of admissions (per patient); 2016	\$7,802	-	-	-
				Cost of ambulatory encounters (per patient); 2016	\$12,978	-	-	-
				Cost of prescriptions (per patient); 2016	\$510	-	-	-
Pogna, 2021	EU-5 and the Netherlands	ITP	148	Mild bleeding events (cost per event); 2020	€3,350	-	-	31.3- 54.3
				Moderate bleeding events (cost per event); 2020	€19,151	-	-	21.5- 41.7
				Severe bleeding events (cost per event); 2020	€34,172	-	-	35.3- 57.2
Roussotte, 2022	France	Secondary ITP	90	-	-	-	6	
Mahevas, 2016	France	Refractory ITP	37	-	-	24	-	
Lucchesi, 2023	Italy	Primary ITP	69	-	-	52	-	
Lozano, 2021	Spain	Primary ITP	67	-	-	-	37.3	
Khair, 2021	UK	ITP	58	-	-	-	79.31	
Wall, 2023	Canada	Primary and secondary ITP	46	-	-	11	-	

Abbreviations- HCRU, Healthcare Resource Utilization; ICU, Intensive Care Unit; EU-5, European Union (France, Germany, Italy, Spain & UK); ITP, Immune Thrombocytopenia; USA, United States of America; UK, United Kingdom

Humanistic Burden

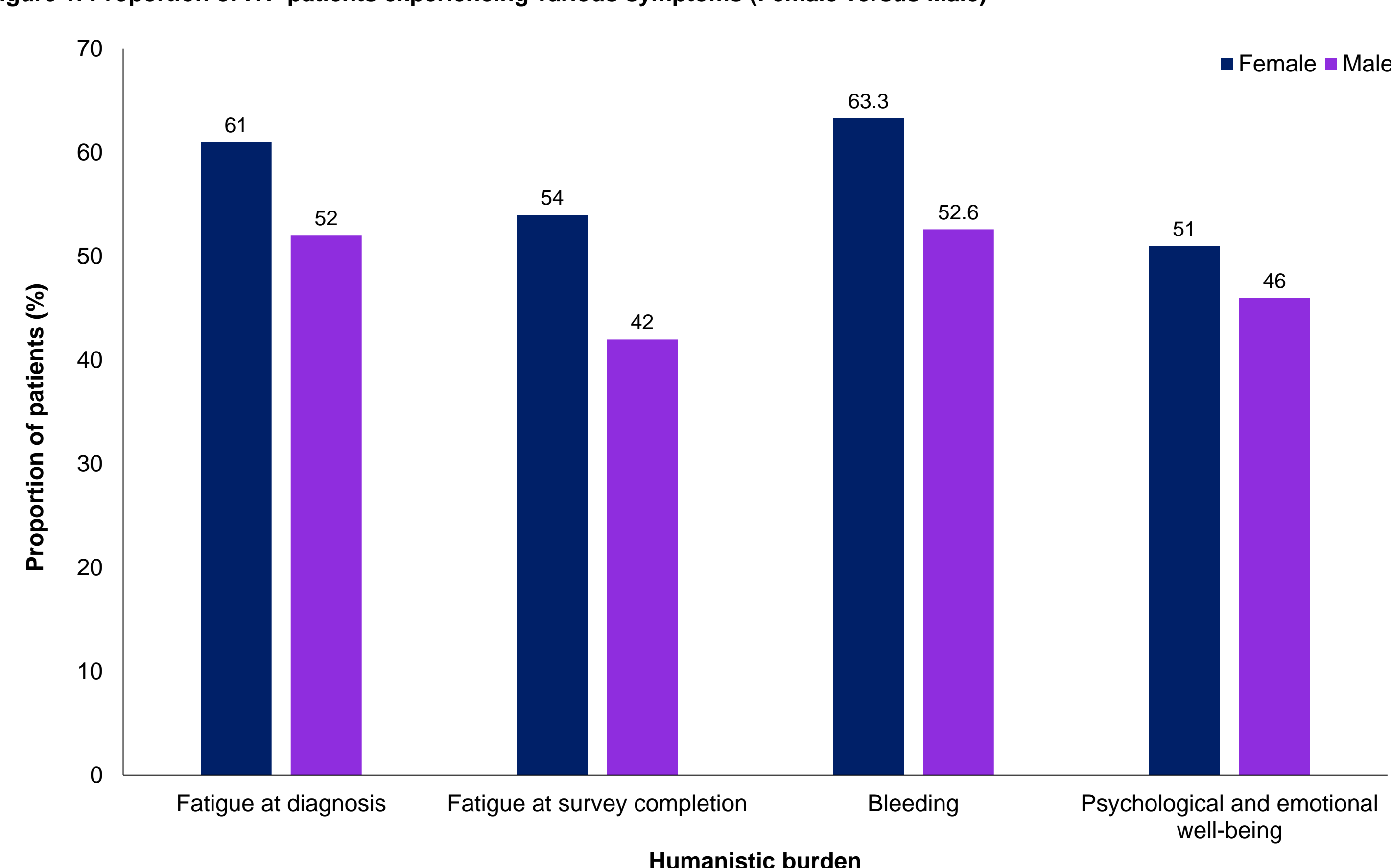
- In the included studies, generic tools such as Short form 36 V1 (SF-36 V1), Functional assessment of chronic illness therapy-fatigue (FACIT-F), Multidimensional Fatigue Inventory (MFI), Patient Health Questionnaire-9 (PHQ-9), and Fatigue Impact Scale (FIS) were utilized to evaluate QoL, while one study employed the disease-specific tool, ITP Life Quality Index (ILQI) questionnaire.
- The SF-36 V1 was the commonly used tool for QoL assessment in the studies included.
- Among ITP patients, fatigue was the most commonly reported symptom. The severity of fatigue was assessed using scales such as FIS, MFI, FACIT-F and PHQ-9.
- Yang et al. study found that ITP patients demonstrated lower FACIT-F scores compared to the general population (37.54 versus 44.42; p<0.001), indicating higher severity of fatigue. (6)
- The studies observed a negative impact of ITP on the patients' emotional and psychological well-being, indicating a high impact of disease on their mental health.
- The iWISH study, utilized the ILQI questionnaire, highlighted the impact of ITP on patients' QoL, particularly affecting energy levels, physical activity, and daily tasks. Additionally, over half of the patients reported frequent disruptions in their social lives due to ITP. (7)

Table 3. Patient-reported outcome measures (PROMs) based humanistic burden data

Study name	Country	Population type	Subgroup	QoL scale	Sub-scale/ domains	Patients analyzed	Time points	Proportion of patients (%)	Mean*
Caocci, 2022	Italy	Primary ITP	Patient received splenectomy	SF-36 V1	RE	26	Follow-up of 10 years	-	58
Efficace, 2016	Italy	Primary ITP	Overall	SF-36 V1	RE	400	-	-	62.7
			Overall	MFI	Fatigue	400	-	-	11.41
Yang, 2020	China	ITP	Overall	SF-36 V1	RE	203	-	-	56.2
			Overall	FACIT-F	-	203	-	-	37.54
Rovó, 2022	Multi-country**	Primary ITP	Primary ITP	Impact on daily life	Emotional wellbeing	46	-	17	-
Cooper, 2021 (b)	Multi-country***	ITP	ITP	Psychologic al/emotional well-being	Score 5-7	1507	-	49	-
Cooper, 2023	Multi-country****	ITP	-	PHQ-9	Emotional wellbeing	1017	-	54	-
			Overall	PHQ-9	Fatigue	1018	At diagnosis	54	-
Mitchell, 2019	UK	ITP	Overall	FIS	FIS ≥ 40	62	2009-2016	39	-
Yang, 2020	China	ITP	Overall	FACIT-F	-	203	-	-	37.54
Cooper, 2023	USA	Primary ITP	Overall	Interviews	Fatigue	17	-	23.5	-
Lucchesi, 2023	Italy	Primary ITP	Overall	Survey	Fatigue	66	-	33	-
McDonald, 2021	UK	ITP	Overall	Survey	Fatigue	31	-	43	-

Abbreviations- QoL: Quality of Life; ITP: Immune Thrombocytopenia; RE: Role Emotional; USA: United States of America; UK: United Kingdom; SF-36: Short Form 36; MFI: Multidimensional Fatigue Inventory; PHQ-9: Patient Health Questionnaire-9; FIS: Fatigue Impact Scale; FACIT-F: Functional Assessment of Chronic Illness Therapy-Fatigue.
* SF-36 v1: score 0 (maximum disability) to 100 (no disability); FACIT-F: score 0 (most fatigue) to 52 (no fatigue); MFI: score 4 (low fatigue) to 20 (high fatigue)
** Switzerland, Austria and Belgium
*** Canada, China, Colombia, Egypt, France, Germany, India, Italy, Japan, Spain, Turkey, UK and USA
**** Australia, China, Egypt, France, Germany, Italy, India, Israel, Japan, Norway, South Korea, Taiwan, Spain, USA and UK

Figure 1. Proportion of ITP patients experiencing various symptoms (Female versus Male)



Source: Cooper, 2021 (a and b) and Piel-Julian, 2018 (7,8,9)

References

1. Vaillant J GN. ITP-Immune Thrombocytopenic Purpura. StatPearls; 2023.
2. Ni J, et al. Journal of Medical Imaging and Health Informatics. 2015;5.
3. Weycker D, et al. Journal of Medical Economics. 2020;23(2):184-92.
4. Roussotte M, et al. Rheumatology (Oxford). 2022;61(9):3627-39.
5. Pogna EA, et al. Hematology. 2021;26(1):860-9.
6. Yang R, et al. Indian Journal of Hematology and Blood Transfusion. 2020;36(1):104-11.
7. Cooper N, et al. American Journal of Hematology. 2021;96:199-207 (a).
8. Cooper N, et al. American Journal of Hematology. 2021;96(2):188-98 (b).
9. Piel-Julian ML, et al. Journal of Thrombosis Haemostasis. 2018;16(9):1830-42.
10. Rovó A, et al. PLoS One. 2022;17(4):e0267342.
11. Sokal A, et al. British Journal Haematology. 2022;196(5):1262-70.
12. Palandri F, et al. Thrombosis Research. 2020;185:88-95.
13. Lozano ML, et al. Blood Cells Molecules and Diseases. 2021;86:102505.
14. Liang Y, et al. Current Medical Research and Opinion. 2021;37(8):1315-22.
15. Kuter DJ, et al. Blood. 2023;142(Supplement 1):3953-.
16. Efficace F, et al. American Journal of Hematology. 2016;91(10):995-1001

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Conflict of Interest

Isabelle Lundqvist, Aditi Kataria, Shaun Walsh, Roberto Abi Rached, Kalitsa Filioussi, Vilas Maroti Belekar and Nitin Kaushik are employees of Novartis.



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