Isabelle Lundqvist | isabelle.lundqvist@novartis.com

PCR260

Utility Estimates for Health States Associated with Immune Thrombocytopenia: A Systematic Literature Review

Isabelle Lundqvist[1], Kalitsa Filioussi[2], Shaun Walsh[3], Aditi Kataria[4], Vilas Belekar[4]

[1]Novartis Sverige AB, Kista, Sweden; [2]Novartis Farma, Milan, Italy; [3]Novartis Ireland Ltd, Dublin, Ireland; [4]Novartis Healthcare Pvt. Ltd., Hyderabad, India

KEY FINDINGS & CONCLUSIONS

- Overall findings from the SLR showed that HRQoL declined with increasing ITP severity, with the greatest impact observed for severe bleeding (ICH and GI).
- Bleeding episodes were found to be a stronger determinant of lower utilities than platelet count alone.
- The EQ-5D measurement was the most commonly used tool for utility estimation but often lacked sensitivity to ITP-specific symptoms (fatigue and emotional burden) and failed to capture impact of platelet count variations on HRQoL.
- There is a need for validated disease-specific instruments to better capture full impact of ITP on patient HRQoL and support more accurate utility estimation and treatment.

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INTRODUCTION

- Immune thrombocytopenia (ITP) is an autoimmune disorder characterized by low platelet counts, increased bleeding risk, fatigue, and a reduced health-related quality of life (HRQoL), with an incidence of approximately 3.3 per 100,000 people.[1-3]
- Despite multiple treatment options, managing chronic ITP in adults remains challenging due to limited long-term responses and remission rates, as well as substantial HRQoL burden from both the disease and its therapies.[4]
- To assess new ITP therapies, it is imperative to quantify their value using utility estimates within economic models submitted to reimbursement agencies.
- A systematic literature review was conducted to identify and summarize studies reporting health-states utility values associated with ITP (primary/relapsed/refractory), which serve as key inputs for economic modeling.

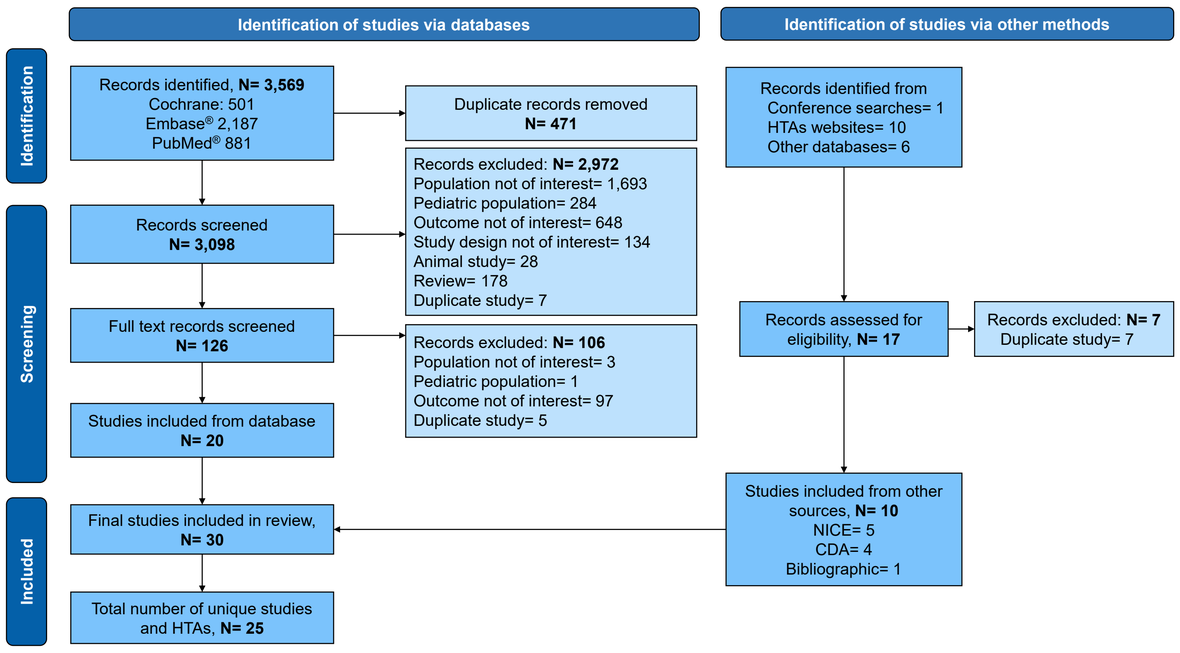
METHODS

- Systematic searches were conducted in key biomedical databases (Embase®, MEDLINE®, and the Cochrane Library) from inception to February 12th, 2025. Only English-language publications were included.
- Relevant conference proceedings and health technology assessment (HTA) reports were also searched.
- Studies reporting utility/ disutility data for adults with ITP were considered for inclusion.
- Screening of studies and data extraction were performed by two independent reviewers. Any discrepancies were resolved by a third independent reviewer.
- The methodological quality and risk of bias of included studies were assessed using the National Institute for Health and Care Excellence-Health State Utility-Values (NICE-HSUV).

RESULTS

- Of the 3,098 records obtained from the database searches, 25 studies met the inclusion criteria including 17 published studies (comprising 8 original research studies and 9 economic evaluations) and 8 HTA reports (**Figure 1**).

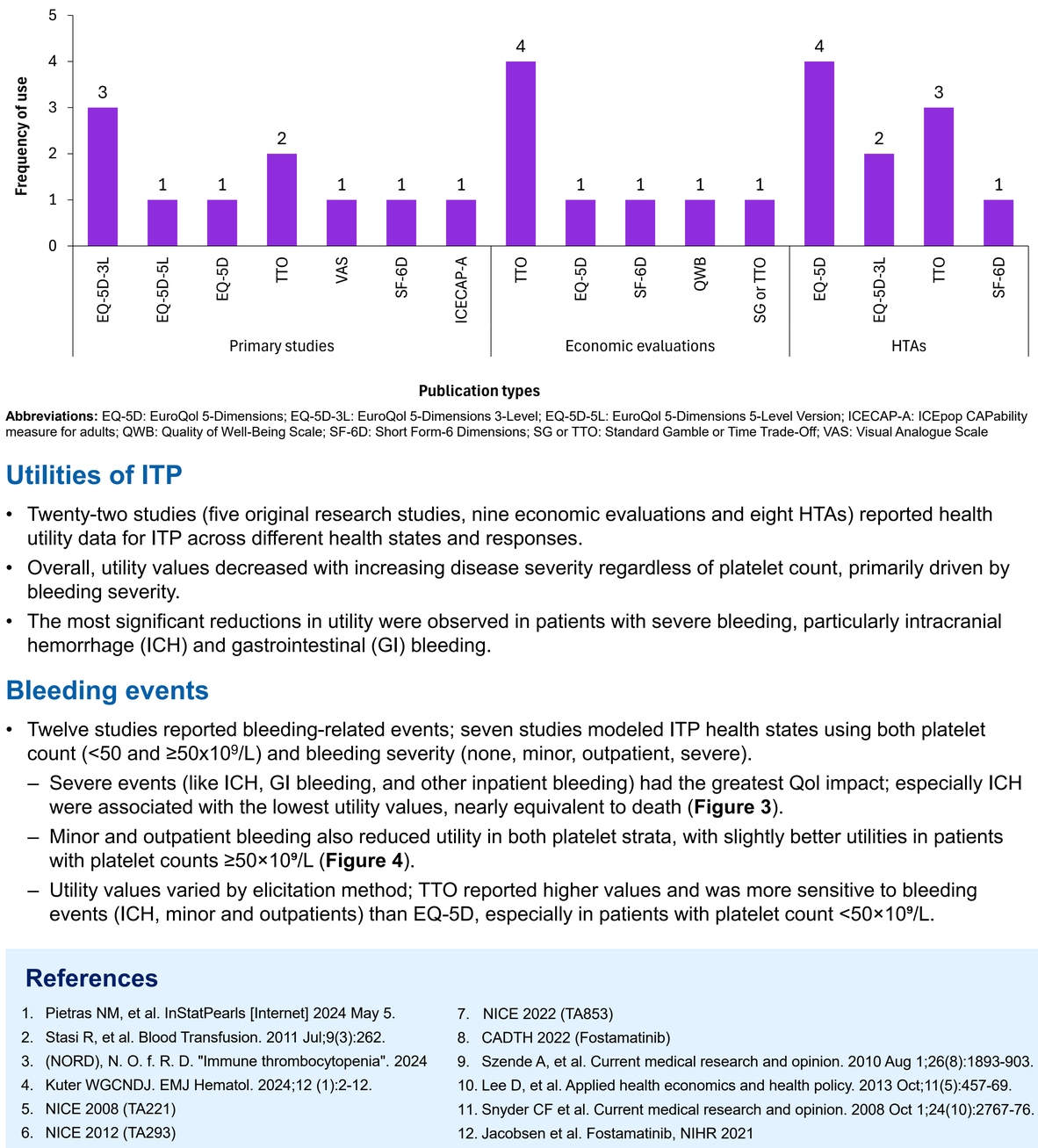
Figure 1: PRISMA flow diagram for the SLR



Abbreviations: CDA: Canadian Drug Agency; HTA: Health Technology Assessment; NICE: National Institute for Health and Care Excellence; PRISMA: Preferred Reporting Items for Systematic Reviews and Meta-Analyses; SLR: Systematic Literature Review.

- Majority of studies were conducted in Europe (n=9), followed by Canada (n=6), USA (n=4), China (n=2), and multi-country (n=2).
- Mean age of patients ranged from 35 to 54 years, with a predominance of females.
- EuroQol 5-dimensional (EQ-5D, including both EQ-5D-3L and EQ-5D-5L) was the most common utility elicitation tool in primary studies (n=5) and HTAs (n=6), while Time Trade-Off (TTO) was used most frequently in economic evaluations (n=4) (**Figure 2**).
- Most of studies focused on the second-line therapy (2L) (n=8), followed by ≥2L (n=6).

Figure 2: Overview of methods used to elicit utility values across studies



Abbreviations: EQ-5D: EuroQol 5-Dimensions; EQ-5D-3L: EuroQol 5-Dimensions 3-Level; EQ-5D-5L: EuroQol 5-Dimensions 5-Level Version; ICECAP-A: ICEpop CAPability measure for adults; QWB: Quality of Well-Being Scale; SF-6D: Short Form-6 Dimensions; SG or TTO: Standard Gamble or Time Trade-Off; VAS: Visual Analogue Scale

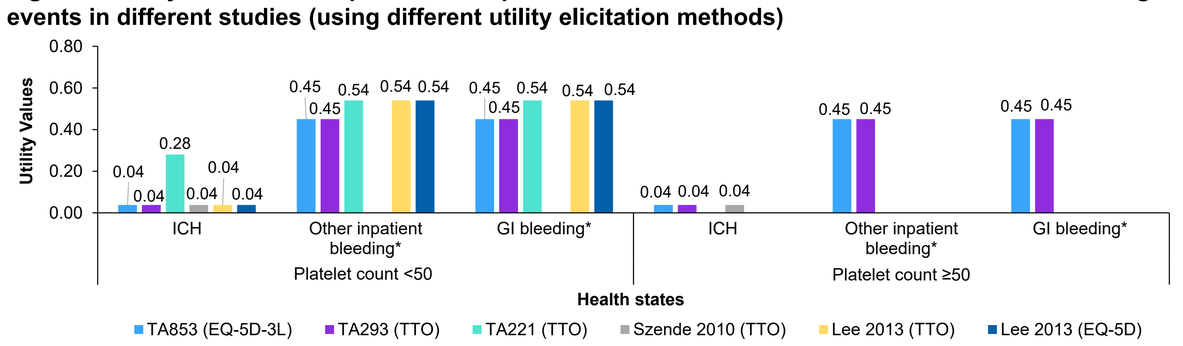
Utilities of ITP

- Twenty-two studies (five original research studies, nine economic evaluations and eight HTAs) reported health utility data for ITP across different health states and responses.
- Overall, utility values decreased with increasing disease severity regardless of platelet count, primarily driven by bleeding severity.
- The most significant reductions in utility were observed in patients with severe bleeding, particularly intracranial hemorrhage (ICH) and gastrointestinal (GI) bleeding.

Bleeding events

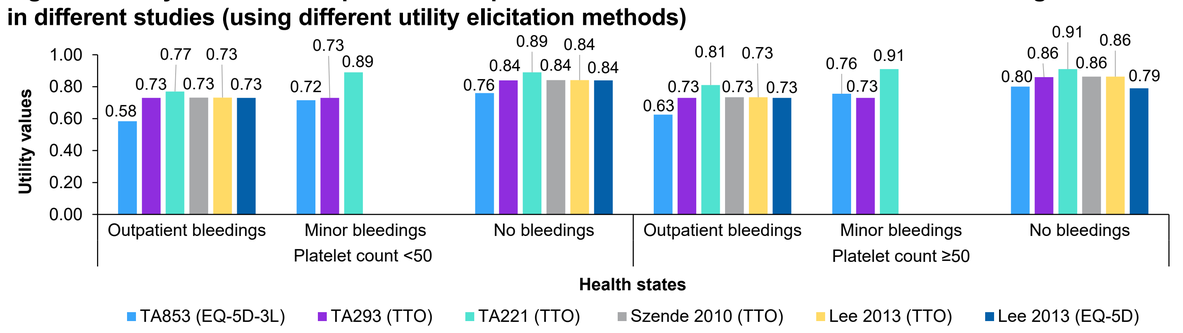
- Twelve studies reported bleeding-related events; seven studies modeled ITP health states using both platelet count (<50 and ≥50x10⁹/L) and bleeding severity (none, minor, outpatient, severe).
  - Severe events (like ICH, GI bleeding, and other inpatient bleeding) had the greatest QoL impact; especially ICH were associated with the lowest utility values, nearly equivalent to death (**Figure 3**).
  - Minor and outpatient bleeding also reduced utility in both platelet strata, with slightly better utilities in patients with platelet counts ≥50×10⁹/L (**Figure 4**).
  - Utility values varied by elicitation method; TTO reported higher values and was more sensitive to bleeding events (ICH, minor and outpatients) than EQ-5D, especially in patients with platelet count <50×10⁹/L.

Figure 3: Utility values for ITP patients with platelet counts <50 and ≥50×10⁹/L across severe bleeding events in different studies (using different utility elicitation methods)



Abbreviations: EQ-5D-3L: EuroQol 5-Dimensional,3-Level Version; GI: Gastrointestinal; ICH: Intracranial Hemorrhage; ITP: Immune Thrombocytopenia; TTO: Time Trade-Off
* Assumption based utility values reported in HTAs and studies

Figure 4: Utility values for ITP patients with platelet counts <50 and ≥50×10⁹/L across bleeding severities in different studies (using different utility elicitation methods)



Abbreviations: EQ-5D-3L: EuroQol 5-Dimensional; 3-Level Version; ITP: Immune Thrombocytopenia; TTO: Time Trade-Off

Platelet count

- Seven studies reported health states utilities by platelet count.
- Modest HRQol improvements were observed when platelet count was above >50×10⁹/L, but difference in utilities between <30 and 30–50×10⁹/L were minimal, suggesting a persistent symptom burden (e.g., fatigue, anxiety, and bleeding risk) (**Table 1**).
- A US cross-sectional study reported that EQ-5D utility values remained stable across platelet levels (p=0.172), highlighting the limited sensitivity of EQ-5D to detect HRQoL variation by platelet level (**Table 2**).

Table 1: Utility values based on platelet count across health states

Study	Scale	Response (>50×10⁹/L)	Partial response (30–50×10⁹/L)	Non-response (<30×10⁹/L)	Controlled platelet count (≥30x10⁹/L)
Fostamatinib: NIHR	EQ-5D + TTO	0.835	0.80	0.80	-
Fostamatinib: ERG	EQ-5D	0.794	0.762	0.762	-
Fostamatinib: CDA	EQ-5D	0.815	0.82	0.81	-
Romiplostim (TA221)	EQ-5D	0.794	-	0.762*	-
Eltrombopag (TA293)	SF-6D	0.73	0.70	0.69	0.73

Abbreviations: EQ-5D: EuroQol 5-Dimensional; SF-6D: Short Form-6 Dimensions; TTO: Time Trade-Off; * Platelet count <50×10⁹/L

Table 2: Utility values based on platelet count levels

Study	Scale	Platelet count (x10⁹/L)					
		≤10K	11-29K	30-49K	50–99k	100-149K	≥150k
Snyder, 2008	EQ-5D-3L	0.75	0.83	0.82	0.82	0.79	0.82

Abbreviation: EQ-5D-3L: EuroQol 5-Dimensions, 3-Level Version

Disutility values associated with ITP

- Grade 3-4 adverse events associated with TPO-RAs, rituximab and immunosuppressants had disutilities between -0.10 and -0.40, while non-serious events across therapies had a consistent disutility of -0.10.
- Severe events like ICH (-0.766) and GI bleeding (-0.354) were associated with substantial disutilities, reflecting significant HRQoL impairment.

Table 3: Disutility values associated with ITP treatments and events

Study	Items	Disutility
Avatrombopag (TA853)	Minor bleeding	-0.044
	Outpatients bleeding	-0.1758
Jacobsen, 2020	ICH	-0.766
	GI/inpatient bleeding	-0.354
	Rescue event	-0.181 (0)$
	Outpatient bleeding (<30×10⁹/L)	-0.072
	Outpatient bleeding (≥50×10⁹/L)	-0.07
	Carer disutility: Severe disability post ICH (<30x10⁹/L; 30-50x10⁹/L; >50x10⁹/L)	-0.162
	Disutility associated with adverse reactions – fostamatinib	
	Diarrhea	-0.044
	Hypertension	-0.0575 (-0.038)$
	Nausea	-0.062 (-0.054)$
	Fatigue	-0.049 (-0.056)$
	Anemia	-0.32 (-0.22)$
	Dizziness (with fall)	-0.12
	Abdominal pain	-0.069
	Pyrexia	-0.11

Abbreviations: GI: Gastrointestinal; ICH: Intracranial Hemorrhage
$ ERG preferred values

References

1. Pietras NM, et al. InStatPearls [Internet] 2024 May 5.
2. Stasi R, et al. Blood Transfusion. 2011 Jul;9(3):262.
3. (NORD), N. O. f. R. D. "Immune thrombocytopenia". 2024
4. Kuter WGCNDJ. EMJ Hematol. 2024;12 (1):2-12.
5. NICE 2008 (TA221)
6. NICE 2012 (TA293)
7. NICE 2022 (TA853)
8. CADTH 2022 (Fostamatinib)
9. Szende A, et al. Current medical research and opinion. 2010 Aug 1;26(8):1893-903.
10. Lee D, et al. Applied health economics and health policy. 2013 Oct;11(5):457-69.
11. Snyder CF et al. Current medical research and opinion. 2008 Oct 1;24(10):2767-76.
12. Jacobsen et al. Fostamatinib, NIHR 2021

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Disclosures

Isabelle Lundqvist, Kalitsa Filioussi, Shaun Walsh, Aditi Kataria and Vilas Belekar are employees of Novartis

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