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A Systematic Literature Review on Economic Evaluations and Cost-Effectiveness of Second-Line Treatment Options for Immune Thrombocytopenia

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KEY FINDINGS & CONCLUSIONS

- Data used in models is of a short-time frame, limiting insights into the long-term economic impact in ITP and potentially overlooking ongoing treatment costs, subsequent therapy expenses, and costs from adverse events.
- Across all the evaluations, TPO-RAs, EPAG and ROMI were found to produce favorable results when compared mostly with standard of care (SoC).
- Indirect treatment comparison of ITP treatments are difficult due to multiple data gaps.
- Economic evaluations presenting an incomplete view on impact of bleeding in patient's quality of life (QoL) and subsequent QALY due to lack of long-term data.

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INTRODUCTION

- Immune thrombocytopenia (ITP) is an autoimmune disease, causing low platelet counts, leading to bleeding risks. The available treatments focus on avoiding bleeding events, typically with corticosteroids or IVIg, and thrombopoietin receptor agonists (TPO-RAs) but later treatments vary and can be costly primarily due to hospitalization required to manage bleeding events, and drug costs and the chronicity of the disease.[1, 2]
- This systematic literature review aimed to identify and critically evaluate the published evidence on cost-effectiveness of second-line treatment options for adult patients with ITP.

METHODS

- Scientific databases including Embase®, MEDLINE®, Cochrane library, and PubMed were searched for relevant publications from inception till 10th February 2025.
  - Hand search of various HTA databases was conducted to retrieve published reports on interventions of interest in ITP.
  - Screening of studies was conducted by two independent reviewers. Any differences were reconciled by the third reviewer.
  - Data on patient characteristics, model specifications, utility and cost inputs, Quality Adjusted Life Years (QALYs), Life Years and Incremental Cost-effectiveness Ratios were extracted from the included studies.

RESULTS

- A total of 50 economic evaluation publications on ITP were identified, of which included 21 HTA documents, 21 journal articles, and 8 conference abstracts.
- These were primarily from Europe (22) and North America (18), followed by Asia (4), Oceania (3), South America (2), and the Middle East (1).
  - Among the 20 evaluations on second-line ITP, the majority were from North America (11), followed by Europe (5), Asia (3) and one from Oceania **(Table 1).**
  - Whereas second-line and above ITP was evaluated in eight studies- Europe (5), and one each from Asia, Oceania, and South America.
  - Remaining publications reported results on ITP in first-line or did not clearly mention the line-of-therapy.
- Out of total second-line ITP publications, eleven were cost effectiveness analysis (CEA), seven were cost utility analysis (CUA), one was a cost minimization analysis (CMA) and one was budget impact analysis (BIA).
  - Among second-line and above economic evaluations three were CEA, two were CUA, and one each were CMA, BIA, and other.
- Recent publications evaluated TPO-RAs alone or in different sequences of therapy **(Table 1).**
  - There is a significant increase in studies post-2011 with broader geographic diversity that including Asian countries.

Table 1. Chronological distribution of economic evaluations on ITP in 2L and beyond

Type of assessment	Total	<2000	2000-2010	2011-2020	>2020
2L					
CEA	11	-	Ireland	UK , USA , India, Japan	USA, Japan, China
CUA	7	USA	Scotland, Canada	Canada	Canada , China
CMA	1	-	-	-	USA
Other	1	-	-	-	Italy*
Interventions		• Immunoadsorption column	• Eltrombopag • Romiplostim • Romiplostim + SC	• Eltrombopag • Rituximab • Eltrombopag + SoC; • Rituximab in Sequence	• Avatrombopag • Eltrombopag • Fostamatinib • Romiplostim • Splenectomy • rhTPO + Rituximab • Sequence (Splenectomy -Rituximab – TPO-RA)
2L and beyond* **					
CEA	3	-	-	UK , France	-
CUA	2	-	-	Ireland	-
CMA	1	-	Australia	-	-
Other	1	-	-	Spain*	-
BIA	1	-	-	-	Colombia
Interventions			• Eltrombopag	• Eltrombopag • Romiplostim • Rituximab • Romiplostim + SoC	• Romiplostim • Eltrombopag

*Cost-per responder analysis, ***Mixed LOT cohort; no subgroup with>85% patients are 2L and beyond.
Abbreviations: BIA: Budget impact analysis; CEA: cost effectiveness analysis; CMA: cost-maximization analysis; CUA: cost-utilization analysis; SC: supportive care; SoC: standard care; TPO-RA: thrombopoietin receptor agonists.

Economic evaluations in second-line ITP

Model overview

- Details on models used for economic evaluations are scarcely published (**Table 2**).
  - Cost-effectiveness, followed by cost-utility impact is the most often analyzed study type using the Markov models (state-transition, embedded decision tree) are used in most evaluations.
  - Time-horizons used in the models ranged from short-term (26 weeks) to lifetime models (up to 58 years).
- The discount rates typically range between 3–5% per year, aligning with standard health economic guidelines.
- Eltrombopag (EPAG) and romiplostim (ROMI) have the most published economic evaluations, often compared head-to-head or against standard of care.

Treatment-wise economic evaluation, cost-drivers and model results

- Among the various economic analyses conducted, EPAG was evaluated most frequently (n=10), followed by rituximab (n=8) and ROMI (n=7) **(Table 3).**
- ITP treatment costs were the key cost drivers, and dominate across all therapies, followed by hospitalizations and bleeding events..
  - Costs related to adverse events and administration are seldom reported to contribute to overall costs.
  - Bleeding evidence may be biased by awareness of platelet counts, with recurring data gaps across studies.
- TPO-RAs, EPAG and ROMI are favourable as evaluated in various scenarios **(Table 3).**

Table 2. Overview of economic models evaluated in second-line ITP patients

Country	Publication name	Year	Model design	Time horizon	Intervention vs comparator
2L - CEA					
Ireland [3]	NCPE	2010	Markov model	2 yrs	EPAG vs RTX
UK[4]	Boyer	2012	1. Watch and Rescue model 2. Long term model	Watch and Rescue: 26 wks; Long time model: -	EPAG + Standard care vs Standard care
UK[5]	NICE	2014	-	-	RTX
Japan[6]	Kikuchi	2015	Markov model	2 yrs	RTX Sequence 1 (Splenectomy-ROMI) Sequence 2 (Splenectomy-ROMI-RTX) Sequence 3 (Splenectomy-RTX-ROMI)
India[7]	Kapoor	2017	-	-	Rituximab
USA[8]	Tremblay	2018	Cost-consequence model	26 wks	EPAG vs ROMI
UK[9]	NICE	2018	State-transition Markov cohort model	Lifetime	EPAG vs Standard care
USA[10]	Tremblay	2020	Markov model	Lifetime (700 cycles, or 53.70 yrs)	EPAG vs ROMI
China [11]	Rui	2021	Markov model with an embedded decision tree	30 yrs	rhTPO+RTX vs RTX
USA[12]	Goshua	2023	Markov model	20 yrs	Sequence1 (Splenectomy-RTX -TRA) vs RTX - Splenectomy - TRA
Canada[13]	CADTH	2023	-	-	Splenectomy vs RTX, Fostamatinib, or TPO-RA**
2L - CUA					
USA[14]	Kunz	1996	Markov decision-analytic model	10 yrs	Immunoadsorption column vs Splenectomy
Scotland[15]	SMC	2009	Markov model	-	ROMI vs Usual care
Canada[16]	CADTH	2010	Cost-utility analysis	-	ROMI + SC* vs SC* alone
Canada[17]	CDEC	2011	-	-	EPAG vs IVIg
Canada[18]	CADTH	2022	Cost-utility analysis	Lifetime (59 yrs)	Fostamatinib vs Watch and rescue
China[23]	Luo	2024	Decision tree-embedded Markov model	Lifetime (33 yrs)	ROMI vs EPAG
Canada[19]	CADTH	2024	Cost-utility analysis	Lifetime (56 yrs)	Avatrombopag vs Watch and rescue
USA [20]	Patwardhan	2021	52 weeks	52 weeks	EPAG vs ROMI
Italy[21]	Aiello (BIM)	2023	-	-	APAG vs EPAG
2L and beyond					
UK[22]	NICE (CEA)	2011	Cohort-based economic model	Lifetime	SoC + ROMI vs SoC
Ireland[24]	Lee (CEA)	2013	Markov model (Embedded decision tree)	Lifetime	ROMI vs EPAG vs SoC
Australia[25]	González (CEA)	2020	Cost-consequence model	-	EPAG vs RTX
Columbia[26]	Niño (BIA**)	2022	Budget impact model	26 wks	ROMI vs EPAG

*(e.g., corticosteroids and/or immunoglobulins; **romiplostim, eltrombopag, avatrombopag **BIA is mentioned here if the published reports gives details of the analysis and the model used
Abbreviations: APAG: avatrombopag; EPAG: Eltrombopag; SC: Supportive care; SMC: Scottish Medicines Consortium SoC: Standard care; ROMI: Romiplostim; RTX: Rituximab; CEA: Cost Effectiveness Analysis; CUA: Cost Utility Analysis; CMA: Cost Minimization Analysis; BIA: Budget Impact Analysis; SoC: Standard of care; Wks, Weeks; USA, United States of America; Splenec: Splenectomy

Table 3. Model results and treatment-wise economic evaluations (no. of publications)

	Second-line						Second-line and beyond		
Evaluation type	**EPAG**	**ROMI**	**RTX**	**APAG**	**Fosta.**	**Splenect.**	**EPAG**	**ROMI**	**RTX**
CEA	6	3	**7**	-	1	3	3	3	2
CUA	2	3	1	1	1	1	1	-	
CMA	1	1	-	-	-	-	1	-	-
BIA	-	-	-	-	-	-	1	1	
Other	1	-	-	1	-	-	1	-	1
Model result									
Favorable / acceptable*	• vs SoC;*** post splenectomy • vs ROMI	• vs placebo • vs SC	• vs SoC	• vs EPAG • vs RTX, Placebo	• vs Placebo (Watch and Rescue)		• vs ROMI; post splenectomy • vs RTX • vs ROMI	• vs RTX • vs EPAG, vs SoC • vs SoC	
Unfavorable**	• vs RTX • vs SoC; with or without splenectomy	• vs EPAG					• vs SoC	• vs EPAG	

Abbreviations: APAG: avatrombopag; EPAG: Eltrombopag; SC: Supportive care; SMC: Scottish Medicines Consortium SoC: Standard care; ROMI: Romiplostim; RTX: Rituximab; Fosta: Fostamatinib
*where publication has concluded that intervention is cost-effective, dominant, or is more effective but costly.
**where results of publication mark the intervention as non-cost-effective, or not-recommended based on their economic-evaluation analysis.
***SoC included azathiopurine, corticosteroids, anti-D immunoglobulins, IVIg, rituximab, mycophenolate mofetil, cyclosporine.

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Disclosures

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