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POSTER ABSTRACTS

901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

Cost-Effectiveness of Caplacizumab in the Warranty Program in Immune Thrombotic Thrombocytopenic Purpura in the USA

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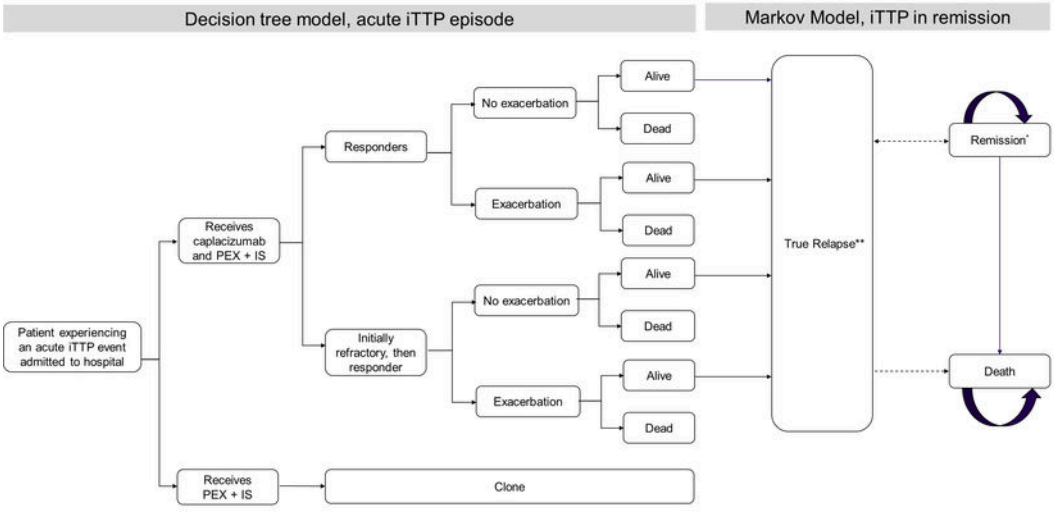
Background
Immune thrombotic thrombocytopenic purpura (iTTP) is a rare, life-threatening thrombotic microangiopathy. Caplacizumab is a humanized nanobody that inhibits formation of microthrombi in iTTP by disrupting binding of von Willebrand factor to platelets. It is the only treatment approved in combination with plasma exchange therapy (PEX) and immunosuppression (IS) for the treatment of iTTP. The United Kingdom National Institute for Health and Care Excellence (NICE) committee reviewed cost-effectiveness of caplacizumab. The committee concluded that addition of caplacizumab to PEX+IS is cost-effective and recommended that the drug be reimbursed in the UK. The Sanofi Promise Warranty Program ® (SPWP) was introduced in January 2023 in the US. It refunds the full cost of caplacizumab to the hospital when treatment is discontinued due to non-response defined as refractoriness (patient with platelet counts $<50 \times 10^9$/L after 4 days of combined treatment with caplacizumab and PEX+IS; up to 6 doses are refunded), or exacerbation (new drop in platelet count after initial platelet count normalization [$>150 \times 10^9$/L], necessitating re-initiation of PEX after ruling out any causes for the drop in platelet count other than iTTP pathophysiology, up to 12 pre-exacerbation doses are refunded).
Objectives
To evaluate the cost-effectiveness of adding caplacizumab to PEX+IS from a US perspective within the context of SPWP.
Methods
The NICE model was adapted to the US setting by replacing UK costs with US costs and discount rates. All clinical assumptions and structure remain unchanged. Clinical inputs were derived from the HERCULES trial (NCT02553317) intent-to-treat population (mean age: 46 years; females: 69%, exacerbation rates: 4.17% during double blind phase and 12.50% in total; refractoriness: 0%). The model consisted of a 3-month decision tree, which captured patient treatment and outcomes over the acute iTTP episode, followed by a long-term Markov model (time horizon: up to 55 years; 3-monthly cycles; *Figure 1*). An annual discount rate of 3% was applied for both costs and quality-adjusted life-years (QALYs).
Under the SPWP, the cost of caplacizumab was assumed to be $0 for refractory patients and those who exacerbate early, no discount was applied for patients who experience exacerbations after drug discontinuation, however, clinical outcomes were captured.
Results
Prior to the SPWP, caplacizumab addition to PEX+IS delivered a gain of 1.75 QALYs (2.96 life-years [LYs]) as compared with PEX+IS alone, at an increased lifetime cost of $256,000. The incremental cost effectiveness ratio (ICER) was $146,350 per QALY and $86,420 per LY gained (*Table 1*).
An estimated 4.17% of treated patients qualified for the SPWP. With this outcomes-based program, the ICER improved to $138,480 per QALY and $81,780 per LY gained, respectively (*Table 1*).
Conclusion
Based on these analyses using the UK NICE model adapted with US costs, caplacizumab is cost-effective considering WTP threshold of $150,000 to $200,000 and has improved cost-effectiveness when the warranty program is taken into account. Outcomes based programs can improve the cost effectiveness of a treatment in addition to mitigating concerns of perceived uncertainty of efficacy in treating patients with iTTP in the US.

Disclosures Sullivan: *Sanofi:* Consultancy. **Chaturvedi:** *Sanofi:* Other: Advisory board participation; *Sobi:* Honoraria; *Takeda:* Other: Advisory board participation; *Sanofi Genzyme:* Consultancy; *Alexion:* Consultancy, Other: Advisory board participation. **Gautam:** *Sanofi:* Current Employment. **Arnaud:** *Sanofi:* Current Employment.

Figure 1. Model description.



iTTP, immune thrombotic thrombocytopenic purpura.

*The remission state includes patients with no chronic conditions, patients with cognitive impairment, patients with neuro-psychological impairment, and patients with both cognitive and neuro-psychological impairment.

**The true relapse health state is included in the model using payoff approach. Outcomes from the relapse and the period in remission following the relapse are applied as a one-off lump sum. Therefore, transitions from true relapse back to remission are not modelled explicitly. Due to payoff approach, outcomes following relapse are not constrained by time horizon in standard manner. Treatment costs for the acute episode also apply to the true relapse state.

Table 1. Results of the base case analysis, and the warranty program.

	PEX+IS	Caplacizumab with PEX+IS
Base case analysis		
Total costs	$358,900	$614,900
Total LYs	17.59	20.55
Total QALYs	7.26	9.01
Incremental Costs		$256,000
Incremental LYs		2.96
Incremental QALY		1.75
ICER per LY		$86,425
ICER per QALY		$146,350
Warranty program		
Total costs	$358,900	$601.140
Total LYs	17.59	20.55
Total QALYs	7.26	9.01
Incremental cost		$242,230
Incremental LY		2.96
Incremental QALY		1.75
ICER per LY		$81,780
ICER per QALY		$138,480

ICER, incremental cost-effectiveness ratio; IS, immunosuppression; LY, life-years; PEX, plasma exchange; QALY, quality-adjusted life years.

Figure 1

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