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COST-UTILITY ANALYSIS OF COMPLEMENT C5 INHIBITORS FOR PATIENTS WITH PAROXYSMAL NOCTURNAL HEMOGLOBINURIA (PNH) IN THAILAND
- A PRELIMINARY ANALYSIS -

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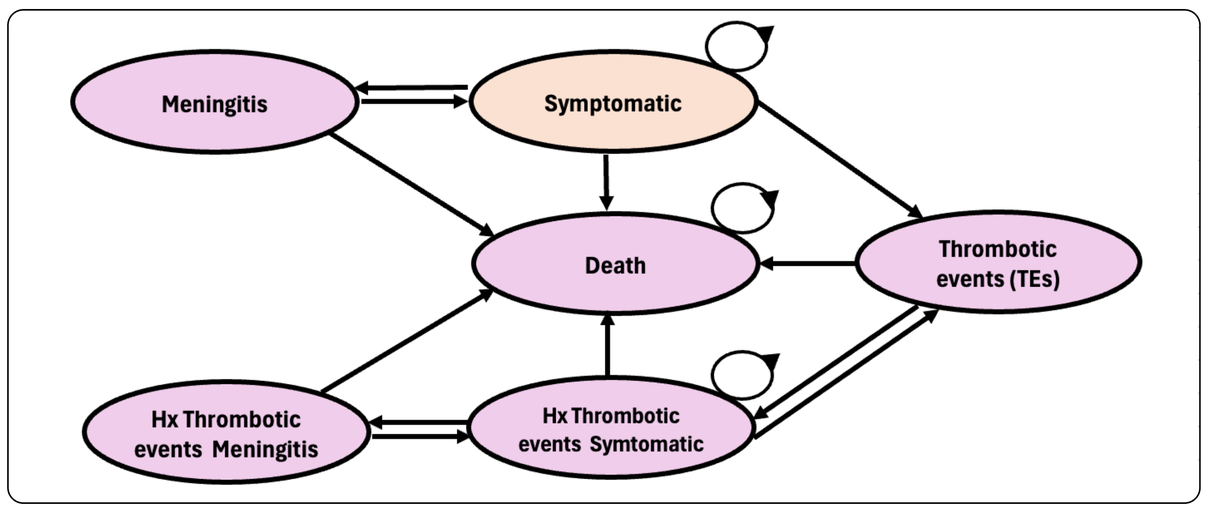
Introduction

- **PNH** is an ultra-rare, chronic intravascular hemolysis disease caused by uncontrolled complement activation.
- **Complement C5 inhibitors,** eculizumab and ravulizumab, may extend survival, improve quality-adjusted life-years (QALYs), and substantially reduce thromboembolic events. However, their cost-effectiveness in Thailand remains uncertain.

Figure 1. A six-state Markov model



Objective

- To evaluate the cost-utility of complement C5 inhibitors for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) in Thailand.

Method

- **A six-state Markov model** compared eculizumab or ravulizumab plus conventional therapy with conventional therapy alone in symptomatic Thai patients with PNH.
- Lifetime costs and health outcomes were estimated from a societal perspective and discounted at 3% annually.
- Model inputs were obtained from literature reviews, secondary data sources, and Thai-specific databases. Treatment efficacy was assumed to be equivalent based on non-inferiority evidence.
- Outcomes included life-years, QALYs, total costs, and ICERs, using a Thai willingness-to-pay threshold of 160,000 THB/QALY.

Table 1. Results, base-case analysis

Treatment	Costs, THB	LYs	QALYs	Incremental Costs	LYs gained	QALYs gained	ICER/LY	ICER/QALY
SoC	5 M	18.02	12.80	**Reference**				
Eculizumab	142 M	21.04	17.25	136 M	3.02	4.45	45.3 M	**30.7 M**
Ravulizumab	224 M	22.84	18.75	218 M	4.82	5.95	45.4 M	**36.8 M**

Results

- Both C5 inhibitors improved survival and QALYs at higher costs than SoC, with **ICERs of THB 30.7 million/QALY and THB 36.8 million/QALY** for eculizumab and ravulizumab, respectively. **Neither treatment was cost-effective** at the Thai WTP threshold.
- Ravulizumab provided the greatest health benefit (5.95 vs 4.45 QALYs gained), whereas eculizumab had the more favorable cost-effectiveness profile, with a lower ICER per QALY gained.
- Both treatments markedly **reduced predicted lifetime thromboembolic** events by approximately 87–88%.

Conclusion

- **Eculizumab and ravulizumab still do not meet the criteria for cost-effectiveness at the Thai willingness-to-pay threshold**, despite substantially reducing thromboembolic events.
- **Further budget impact and feasibility analyses are needed** to support decisions on including complement C5 inhibitors in Thailand's PNH benefit package.

References:
1. Parker C, Omine M, Richards S, Nishimura J, Bessler M, Ware R, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. Blood. 2005;106(12):3699–709
2. Hillmen P, Young NS, Schubert J, Brodsky RA, Socie G, Muus P, et al. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. N Engl J Med. 2006;355(12):1233–43.
3. Lee JW, Sicre de Fontbrune F, Wong Lee Lee L, Pessoa V, Gualandro S, Fureder W, et al. Ravulizumab (ALXN1210) vs eculizumab in adult patients with PNH naive to complement inhibitors: the 301 study. Blood. 2019;133(6):530–9.