EMICIZUMAB PROPHYLAXIS AMONG HAEMOPHILIA A PATIENTS WITHOUT INHIBITORS IN INDIA

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Recommendations:

- Alternate dose Emicizumab regimen (3 mg/kg every 4 weeks given in sub-cutaneous route) is cost-saving compared to ondemand therapy with FVIII and Low dose FVIII prophylaxis for severe Haemophilia A patients without inhibitors at the current Emicizumab pricing (₹422.2/mg)
- Implementing alternate-dose Emicizumab prophylaxis for Haemophilia A patients without inhibitors in a typical district (n = 14 patients) would require ₹78 lakhs, equivalent to 1.63% of the district's NHM health budget

Key Findings:

For severe Haemophilia A patients without inhibitors:

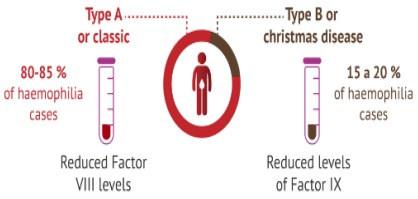
- Cost Effectiveness: Alternate dose of Emicizumab is cost-saving for severe Haemophilia A patients without inhibitors in India*.
- Average annual budget impact (₹) for managing one Haemophilia A patient without inhibitors using alternate dose Emicizumab = ₹ 5,57,140.
- To include alternate dose Emicizumab prophylaxis in the district hospital services, it will require an additional ₹78 lakhs (1.63% of average annual district NHM budget) for 14 patients in a typical district in India.

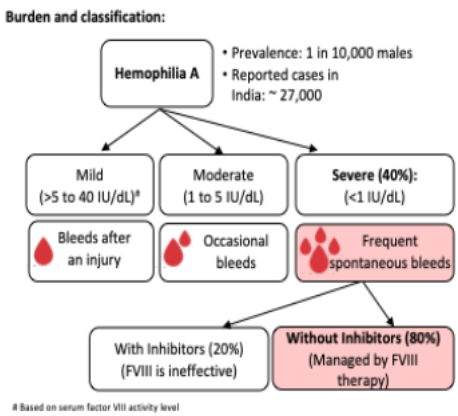
*The incremental cost-effectiveness ratio per QALY is ₹- 97.63 lakhs for an Alternate dose of Emicizumab

Background

- Haemophilia is a genetic bleeding disorder caused by deficiencies in specific clotting factors that are essential for normal blood coagulation. It is inherited in an X-linked recessive pattern, meaning the faulty gene is carried on the X chromosome. Because males have only one X chromosome, they are more commonly affected, while females are typically carriers.
- Emicizumab is monoclonal antibody. It is used to functionally replace Factor VIII and effective in reducing bleeding rate.

PICO	Description of the components of PICO
Population	Severe Haemophilia A patients without Inhibitors
Intervention	Emicizumab prophylaxis • *Standard-dose:* 3 mg/kg weekly × 4 (loading dose) followed by 6 mg/kg every 4weeks • *Alternate dose:* 3 mg/kg every 4 weeks (no loading dose)
Comparator	1. On-demand therapy using SHL FVIII: Body weight × 40 IU/dl × 0.5 2. Low-dose prophylaxis (LDP) with SHL FVIII: 10 -15 IU/kg, 2-3 times per week
Outcome	Incremental cost-effectiveness ratio (ICER) per QALY gained





Results:

Outcome (Discounted at 3%)	SHL FVIII		Alternate dose Emicizumab prophylaxis	
	On-demand	Low dose prophylaxis		
Costs in ₹ (Cr)	4.07	2.32	1.51	
QALYs	19.8	21.3	22.4	
Bleeds	406	75	2	
Comparator*	Comparator		On-demand SHL FVIII	LDP with SHL FVIII
Incremental costs in ₹ (Cr)			-2.55	-0.80
Incremental QALYs			2.6	1.1
ICER / QALY gained ₹			Cost Saving*	Cost Saving#

*ICER for alternate dose Emicizumab vs on-demand SHL FVIII = -97.63 Lacs, # ICER for alternate dose Emicizumab vs LDP with SHL FVIII = -76.16 Lacs

Budget Impact Analysis:

Average annual budget impact (₹) for managing one Haemophilia A patient without inhibitors using alternate dose Emicizumab = ₹ 5,57,140. Assuming a budget of 50 lakhs for patients with rare disease with 50 years life expectancy, the unit price of Emicizumab needs to be reduced to ₹ 75.0/mg.

Outcome	On-demand therapy with SHL FVIII (comparator*)	Low dose prophylaxis with SHL FVIII (comparator*)	Alternate dose Emicizumab
Total four-year budget (2025-2028) (₹) (Cr)	8.77	4.69	3.11
Average annual budget impact (₹) (n=14)	2.19 Cr (4.58%)	1.17 Cr (2.45%)	77.99 Lacs (1.63%)

Conclusion:

- Alternate dose Emicizumab prophylaxis is cost-saving compared to on-demand treatment and low dose prophylaxis with FVIII.
- To meet the rate consistent with rare disease policy of annual per-patient budget of ₹1 lakh, the price of Emicizumab needs to be reduced from ₹422.2/mg to ₹75/mg for managing Haemophilia A without inhibitors.

Reference:

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