Policy Brief

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Cost-effectiveness of Emicizumab prophylaxis versus no prophylaxis among hemophilia A patients with and without inhibitors

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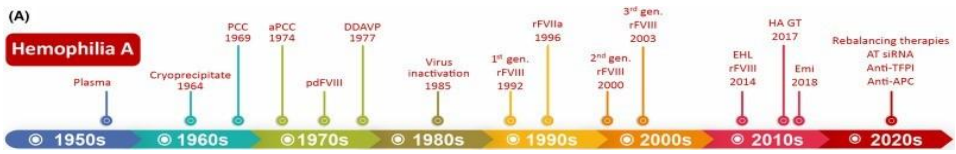
Recommendations

- At the cost effectiveness cut off of 1 GDP (INR 2,31,784) for procurement in public health programmes, the proposed intervention (**Emicizumab compared against HDP among hemophilia A patients**) is highly cost effective.
- Policymakers shall prioritize strategies for affordable pricing of Emicizumab.

Background

- Hemophilia A (HA) affects approximately 30,000 patients in India, with severe cases causing frequent bleeding, reduced quality of life, and complications if untreated.
- Factor VIII (FVIII) prophylaxis is standard treatment but faces challenges like poor adherence and limited access to higher-dose regimens in resource-limited settings.
- Emicizumab, a subcutaneous prophylactic, offers flexible dosing and improved adherence, with trials showing reduced bleeding episodes. (1-4)
- While evaluating the cost-effectiveness of emicizumab prophylaxis versus no prophylaxis among hemophilia A patients with and without inhibitors in India, we identified a pertinent study.
- In this policy brief, we highlight the findings of the published study titled "Cost-effectiveness analysis of emicizumab prophylaxis in patients with haemophilia A in India" and our inference on its relevance to the Indian context. (5)

Fig: 1 Evolution of treatment in Hemophilia A



PICO	Description of the components of PICO
Population	Patients with severe non-inhibitor hemophilia A (HA) in India.
Intervention	Emicizumab prophylaxis.
Comparator	Standard care options, including on-demand therapy (ODT), and low-dose prophylaxis (LPD), intermediate-dose prophylaxis (IDP), and high-dose prophylaxis (HDP) with FVIII.
Outcome	Cost-effectiveness measured through ICER per QALY, reduction in annual bleeding rates (ABR), and improvement in quality of life (QoL).

Key Findings

- Emicizumab prophylaxis demonstrated superior clinical effectiveness, generating higher QALYs compared to ODT, LDP, and IDP with FVIII.
- Cost effectiveness: ICER/ QALY is Rs 27,869/QALY (0.12 times GDP per capita) for Emicizumab compared against HDP among hemophilia A patients However, HDP is not widely prescribed in Indian public healthcare settings.
- Sensitivity analysis indicated Emicizumab cost as the primary driver of ICER values, highlighting the need for price reductions for broader feasibility.

Fig: 2 Pictorial representation of current Prophylaxis and Intervention

Standard care options, including on-demand therapy, and low-, intermediate-, and high-dose FVIII prophylaxis. **(Comparator)**

Emicizumab prophylaxis. **(Intervention)**

Study Findings

- Emicizumab was cost-effective compared to HDP with FVIII, with an incremental cost-effectiveness ratio (ICER) of INR 27,869 per QALY.
- At Willingness to pay thresholds exceeding two times per capita GDP (INR 300,000/QALY), emicizumab was considered cost-effective compared to IDP, LDP, and ODT
- Probabilistic sensitivity analysis showed a 94.7% probability of cost-effectiveness at three times per capita GDP and a 49.4% probability at two times per capita GDP.
- One-way sensitivity analysis identified the cost of emicizumab as the primary driver of ICER. A reduction in drug price would significantly improve its cost-effectiveness across all thresholds.

Conclusion

Emicizumab offers clinical benefits for severe non-inhibitor hemophilia A patients but is cost-effective only compared to HDP, a regimen less commonly used in India. It is not cost-effective against ODT, LDP and IDP.

Policy Implications

- Emicizumab is cost-effective only against HDP.
- HDP is less commonly prescribed in Indian public healthcare settings due to high costs.
- Emicizumab is not cost-effective compared to ODT, LDP & IDP.
- Policymakers shall prioritise strategies for affordable pricing of Emicizumab.