**Young G, Liesner R, Chang T, et al. A multicenter, open-label phase 3 study of emicizumab prophylaxis in children with hemophilia A with inhibitors (HAVEN 2). Blood. 2019;134(24):2127-2138.**

**What is Emicizumab ?** Recombinant, humanized, bispecific monoclonal antibody, that bridges Factor IXa and Factor X restoring the function of missing activated factor VIII in Haemophilia A (HA).

**Background:** Emicizumab has proven efficacy in treating children with HA with inhibitors and has superior efficacy with less target/ new joint bleeds, has a proven safety and is associated with better QOL.

**Method:** Phase 3, multicenter, nonrandomized, open label trial, investigating Emicizumab prophylaxis in children with HA with inhibitors. Emicizumab was administered subcutaneously in a weekly loading dose of 3mg/kg for 4 weeks followed by either 1.5mg/kg every week (group A), 3mg/kg fortnightly (group B) or 6mg/kg every 4 weeks (group C).

# ACADEMIC P.E.A.R.L.S

Pediatric Evidence And Research Learning Snippet

## Emicizumab prophylaxis in children with Hemophilia A with Inhibitors

**Results:** 88 males were enrolled (n-68 in group A, n-10 in group B; n-10 in group C), median age 7 years(1-15 years). Patients had undergone Immune tolerance induction (72%), and were receiving treatment with prophylactic Bypassing agents (75%). Data analysis done at 52 weeks.

•Emicizumab had proven efficacy across all treatment groups, with an Annual Bleeding Rate (ABR) of 0.3 in group A, 0.2 in group B and 2.2 in group C.

•HRQOL improved with Emicizumab prohylaxis- At baseline, the mean proportion of days missed of daycare/school was 0.41 (95% CI, 0.29-0.53); following emicizumab treatment, this decreased to 0.25 (95% CI, 0.01-0.49) at week 13 and remained low at all subsequent time points.

**Safety:** 712 adverse events were reported from 82 of 88 participants which commonly ranged from nasopharyngitis(n-33 participants, 37.5%), local reactions(n-27, 30.7%). No discontinuation of therapy due to adverse event. 21 Serious adverse events were reported and only 1 antibody development was advocated to Emicizumab.

**Conclusion:** Emicizumab is a safe drug in patients with HA with inhibitors and has proven efficacy by decreasing ABR's and positively impacting the HRQOL

**Key message:** Emicizumab is safe in patients with HA in doses ranging from 1.5mg/kg/week to 6mg/kg/month. It has been found to be superior to other bypassing agents (BPAs) in patients with HA and inhibitors with less target/new joint bleed and better HRQOL.

## EXPERT COMMENT

"Emicizumab is a good drug to be used in patients with HA with inhibitors as shown by the above study. This was the first study that compared Emicizumab with other bypassing agents & showed that it can be used in children who have failed prior therapy with them. Its use is perhaps limited in developing countries like India due to its cost but it dramatically improves the HRQOL by reducing hospitalisation, its ease and frequency of administration."

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**Reference :**
Young G, Liesner R, Chang T, Sidonio R et al. A multicenter, open-label phase 3 study of emicizumab prophylaxis in children with hemophilia A with inhibitors. Blood. 2019 Dec 12;134(24):2127-2138. doi: 10.1182/blood.2019001869.