Patient – Quality of Life Results with Hemlibra in Hemophilia A without Inhibitors

This letter contains information you requested about the effect of Hemlibra® (emicizumab-kxwh) on quality of life in people with hemophilia A without factor VIII (factor 8 or FVIII) inhibitors. This letter includes studies with the strongest and most relevant data.

This information is provided only for educational purposes and not for use in treatment decisions. You should talk with your healthcare provider for specific information and advice about your condition, your individual situation, healthcare coverage, and any current or potential treatments.

Glossary

Inhibitors: In hemophilia A, inhibitors are antibodies against infused FVIII clotting proteins. These antibodies make infused FVIII products not effective.

On-demand: On-demand refers to a treatment that is given as needed. For example, when bleeding occurs.

Prophylaxis: Also known as "prophy", it is a treatment given on a regular schedule to prevent bleeds.

Patient reported outcomes: the patient's view of the treatment effect on quality of life and ability to function

What is Hemlibra?

Hemlibra is a medicine approved by the Food and Drug Administration (FDA) for prophylaxis in adults and children with hemophilia A, with or without FVIII inhibitors.[1]

What trials studied Hemlibra for hemophilia A without FVIII inhibitors?

HAVEN 3 studied how safe and how well Hemlibra worked to prevent bleeds in 152 people 12 years or older without FVIII inhibitors.[2] People who used on-demand FVIII before the study were randomly assigned to Hemlibra dosed once weekly, Hemlibra dosed every 2 weeks, or no prophylaxis (continue on-demand FVIII). People who used FVIII prophylaxis before the study switched to Hemlibra dosed once weekly.

People assigned to receive Hemlibra, started at 3 mg/kg once weekly for 4 weeks, followed by a maintenance dose of either 1.5 mg/kg once weekly or 3 mg/kg every 2 weeks (depending on their assigned treatment in the study).[2]

HAVEN 4 studied how safe and how well Hemlibra worked to prevent bleeds in 41 people with or without FVIII inhibitors, of which 36 people did not have a FVIII inhibitors. All people started Hemlibra at 3 mg/kg once weekly for 4 weeks, followed by 6 mg/kg every 4 weeks.

What patient reported outcomes were measured in these trials?

Patient reported outcomes are reported directly from patients about their quality of life and general health.[2] In hemophilia A, these measures help to understand productivity, social and emotional well-being, and ability to do daily activities. The questionnaires have been tested and used by hemophilia experts.

How did Hemlibra affect quality of life in adults with hemophilia A without FVIII inhibitors?

The HAVEN 3 and HAVEN 4 studies measured improvement in the Physical Health score after 25 weeks of treatment.[2] The physical health score assessed:

painful swellings	pain with movement
joint pain	difficulty walking far

In HAVEN 3, adults on Hemlibra prophylaxis maintenance dosing of once weekly or once every 2 weeks scored better in their physical health score compared to adults who took no prophylaxis; however, this was not determined to be statistically significant.[2]

In HAVEN 4, Hemlibra prophylaxis maintenance dosing of once every 4 weeks resulted in improved physical health score compared with their scores before starting Hemlibra.

What was the preference survey?

HAVEN 3 and HAVEN 4 assessed whether people enrolled in the studies preferred Hemlibra or their previous treatment.[2] After 17 weeks, people enrolled in HAVEN 3 and HAVEN 4 were given a survey that asked the following question:

Which of the treatments would you prefer to take as the treatment for your hemophilia? (Mark only one response).

Prefer my old hemophilia treatment (intravenous)
Prefer Hemlibra treatment (subcutaneous)
Have no preference

What were the results of the preference survey in HAVEN 3 and HAVEN 4?[2]

In HAVEN 3, Hemlibra was preferred by 94% of the 95 people completed the preference survey.[2]

Of the subset of 46 people who used infused FVIII prophylaxis before entering HAVEN 3 and switching to Hemlibra, 45 (97.8%) preferred Hemlibra prophylaxis over FVIII prophylaxis.[2]

In HAVEN 4, all 41 people enrolled in the study took the survey, and all preferred Hemlibra.[3]

Patient – Quality of Life Results with Hemlibra in Hemophilia A without Inhibitors References

1. Hemlibra® [package insert]. Genentech, Inc.; South San Francisco, CA. March 2021.

2. Mahlangu J, Oldenburg J, Paz-Patel I, et al. Emicizumab prophylaxis in patients who have hemophilia A without inhibitors [supplementary appendix appears online]. N Engl J Med 2018;379:811-822. https://www.nejm.org/doi/pdf/10.1056/NEJMoa1803550

3. Pipe S, Jimenez-Yuste V, Shapiro A, et al. Emicizumab subcutaneous dosing every 4 weeks is safe and efficacious in the control of bleeding in persons with haemophilia A with and without inhibitors â€“ Results from the phase 3 HAVEN 4 study. Presented at the World Federation of Hemophila World Congress in Glasgow, Scotland; May 20-24, 2018. WFH Oral Presentation.