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Roche receives US FDA nod for blockbuster Hemlibra

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Hemlibra is now the only prophylactic treatment for people with haemophilia A with and without factor VIII inhibitors



Roche recently announced that the US Food and Drug Administration (FDA) has approved Hemlibra (emicizumab-kxwh) for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with haemophilia A without factor VIII inhibitors.

Hemlibra is now the only prophylactic treatment for people with haemophilia A with and without factor VIII inhibitors that can be administered subcutaneously (under the skin) and at multiple dosing options (once weekly, every two weeks or every four weeks).

This approval is based on positive results from the phase III HAVEN 3 and HAVEN 4 studies. Hemlibra prophylaxis led to statistically significant and clinically meaningful reductions in treated bleeds compared to no prophylaxis (primary endpoint) and across all other bleed-related endpoints in the HAVEN 3 study, and showed a clinically meaningful control of bleeding in the HAVEN 4 study.

“Many preventative treatment options for people with haemophilia A without factor VIII inhibitors require intravenous infusions several times a week. Even then, people can still experience bleeds, and there has been a need for more treatment options,” said Michael Callaghan, MD, haematologist, Children’s Hospital of Michigan, Detroit. “The approval of Hemlibra is an important advancement for the entire haemophilia A community, as we now have a new class of medicine for the first time in nearly 20 years. Hemlibra can reduce bleeds, and it offers a new subcutaneous administration once weekly, every two weeks or every four weeks.”

“Today’s approval of Hemlibra reflects our commitment to groundbreaking science and the development of medicines with the potential to redefine the standard of care,” said Sandra Horning, MD, Roche’s Chief Medical Officer and Head of Global Product Development. “Hemlibra is now the only FDA-approved medicine for people with haemophilia A with and without factor VIII inhibitors, based on the efficacy and safety profile demonstrated across four pivotal studies. We want to thank the haemophilia community for their partnership in helping us bring this new option to everyone living with haemophilia A.”

Hemlibra was granted Breakthrough Therapy Designation by the FDA for haemophilia A without factor VIII inhibitors. It was also granted Priority Review, a designation given to medicines that the FDA has determined to have the potential to provide significant improvements in the treatment, prevention or diagnosis of a serious disease. The company’s Marketing

Authorisation Application (MAA) variation for haemophilia A without factor VIII inhibitors, which includes data from the HAVEN 3 and HAVEN 4 studies, is under review by the European Medicines Agency (EMA).

Hemlibra was approved by the FDA in November 2017 for adults and children with haemophilia A with factor VIII inhibitors.