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FDA approves Hemlibra without Factor VIII inhibitors for Hemophilia A

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Hemlibra was approved by the FDA in November 2017 for adults and children with hemophilia A with factor VIII inhibitors. It has been studied in one of the largest pivotal clinical trial programs in people with hemophilia A with and without factor VIII inhibitors, including four pivotal HAVEN studies (HAVEN 1, HAVEN 2, HAVEN 3 and HAVEN 4).

Genentech, a member of the Roche Group has announced that the U.S. 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 hemophilia A without factor VIII inhibitors. Hemlibra is now the only prophylactic treatment for people with hemophilia 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 hemophilia 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, M.D., hematologist, Children’s Hospital of Michigan. “The approval of Hemlibra is an important advancement for the entire hemophilia 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, M.D., chief medical officer and head of Global Product Development. “Hemlibra is now the only FDA-approved medicine for people with hemophilia A with and without factor VIII inhibitors, based on the efficacy and safety profile demonstrated across four pivotal studies. We want to thank the hemophilia

community for their partnership in helping us bring this new option to everyone living with hemophilia A."

In the Phase III HAVEN 3 study, adults and adolescents aged 12 years or older with hemophilia A without factor VIII inhibitors who received Hemlibra prophylaxis once weekly (n=36) or every two weeks (n=35) experienced a 96 percent (95 percent CI: 92.5; 98.0, p<0.0001) and 97 percent (95 percent CI: 93.4; 98.3, p<0.0001) reduction in treated bleeds, respectively, compared to those who received no prophylaxis (n=18). Hemlibra is the first medicine to significantly reduce treated bleeds compared to prior factor VIII prophylaxis, which has been the recommended standard of care, as demonstrated by a statistically significant reduction of 68 percent (95 percent CI: 48.6; 80.5, p<0.0001) in treated bleeds in a prospective intra-patient comparison (n=48) of people who previously received factor VIII prophylaxis in a non-interventional study and switched to Hemlibra prophylaxis. In the single-arm Phase III HAVEN 4 study of adults and adolescents aged 12 years or older with hemophilia A with factor VIII inhibitors (n=5) and without factor VIII inhibitors (n=36), Hemlibra prophylaxis every four weeks (n=41) led to clinically meaningful control of bleeding. The most common adverse reactions occurring in 10 percent or more of people treated with Hemlibra in pooled studies (n=391) were injection site reactions (n=85), headache (n=57) and joint pain (arthralgia; n=59).

Hemlibra is now available to people in the U.S. who have hemophilia A without factor VIII inhibitors. Genentech is committed to helping people with hemophilia A access Hemlibra and offers comprehensive services to help minimize barriers to access and reimbursement. Patients can call (866) HEMLIBRA (436-5427) for more information. For people who qualify, Genentech also offers patient assistance programs through Genentech Access Solutions.

Hemlibra was granted Breakthrough Therapy Designation by the FDA for hemophilia 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. Submissions to other regulatory authorities around the world are ongoing.

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