Supplemental Approvals

Generic Name (Trade Nameâ€"Company)

October 18, 2018

Emicizumab-kxwh injection

(Hemlibra—Genentech)

FDA approves emicizumab-kxwh for hemophilia A with or without Factor VIII inhibitors

Uses/Notes

FDA approved emicizumab-kxwh injection to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients (aged newborn and older) with hemophilia A (congenital factor VIII deficiency) with or without factor VIII (FVIII) inhibitors.

The agent was first approved in 2017 for patients with hemophilia A with FVIII inhibitors.

The current approval was based on two clinical trials: HAVEN 3 (NCT02847637) and HAVEN 4 (NCT03020160). This approval expanded the indication for patients with hemophilia A without FVIII inhibitors and provided for new dosing regimens for patients with and without FVIII inhibitors.

The prescribing information includes a warning that thrombotic microangiopathy and thrombotic events were reported when on average a cumulative amount of greater than 100 U/kg/24 hours of activated prothrombin complex concentrate (aPCC) was administered for 24 hours or more to patients receiving prophylaxis with emicizumab-kxwh. Patients should be monitored for the development of thrombotic microangiopathy and thrombotic events if aPCC is administered. aPCC should be discontinued and emicizumab-kxwh dosing should be suspended if there is evidence of thrombotic microangiopathy or an acute thrombotic event.

The most common adverse reactions reported (incidence ?10%) were injection-site reactions, headache, and arthralgia.

The recommended loading dose is 3 mg/kg by S.C. injection once weekly for the first 4 weeks for all approved prophylactic dosing regimens. In addition to the already approved weekly dose of 1.5 mg/kg, the new maintenance dosing regimens include 3 mg/kg by S.C. injection once every 2 weeks and 6 mg/kg by S.C. injection every 4 weeks.

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