Hemlibra® (emicizumab-kxwh) – Expanded indication

- On October 4, 2018, Genentech announced the FDA approval of Hemlibra (emicizumab-kxwh), for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients ages newborn and older with hemophilia A (congenital factor VIII deficiency) with or without factor VIII (FVIII) inhibitors.
    - Previously, Hemlibra was only approved in adult and pediatric patients with FVIII inhibitors.
- The efficacy of Hemlibra for routine prophylaxis in patients with hemophilia A without FVIII inhibitors was evaluated in two trials, HAVEN 3 and HAVEN 4. HAVEN 3 enrolled 152 adult and adolescent males. HAVEN 4 enrolled 41 adult and adolescent males.
    - In HAVEN 3, patients who received Hemlibra prophylaxis once weekly or every two weeks experienced a 96% (95% CI: 92.5, 98.0; $p < 0.0001$) and 97% (95% CI: 93.4, 98.3; $p < 0.0001$) reduction in annualized bleed rate (ABR) for treated bleeds, respectively, vs. those who received no prophylaxis.
    - In HAVEN-4, Hemlibra prophylaxis every four weeks led to clinically meaningful control of bleeding (ABR 2.6 [95% CI: 1.5, 4.7]) in patients without FVIII inhibitors.
- Hemlibra carries a boxed warning for thrombotic microangiopathy and thromboembolism.
- The recommended loading dose of Hemlibra is 3 mg/kg by subcutaneous (SC) injection once weekly for the first 4 weeks, followed by a maintenance dose of 1.5 mg/kg once a week, 3 mg/kg once every two weeks, or 6 mg/kg every four weeks.
    - The selection of a maintenance dose should be based on healthcare provider preference with consideration of regimens that may increase patient adherence.
    - After proper training in SC injection technique, a patient may self-inject, or the patient's caregiver may administer Hemlibra, if a healthcare provider determines that it is appropriate.