Hemlibra® (emicizumab-kxwh) – New orphan drug approval

- On November 16, 2017, the FDA announced the approval of Genentech's Hemlibra (emicizumab-kxwh), for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A (congenital factor VIII [FVIII] deficiency) with FVIII inhibitors.

- According to the National Institutes of Health, hemophilia affects one in every 5,000 males born in the U.S., approximately 80% of whom have hemophilia A. Patients with hemophilia A are missing a gene which produces FVIII, a protein that enables blood to clot. Patients may experience repeated episodes of serious bleeding, primarily into their joints, which can be severely damaged as a result.

    - Nearly one in three people with severe hemophilia A can develop inhibitors or antibodies to FVIII replacement therapies. The antibody interferes with the effectiveness of currently available treatments for hemophilia.

- Hemlibra is a bispecific factor IXa (FIXa)- and factor X (FX)-directed antibody. It is designed to bring together FIXa and FX, proteins required to activate the natural coagulation cascade and restore the blood clotting process for hemophilia A patients.

- The efficacy of Hemlibra for the treatment of hemophilia A with FVIII inhibitors was demonstrated in two clinical studies, HAVEN 1 and HAVEN 2. HAVEN 1 enrolled 109 patients ≥ 12 years of age. HAVEN 2 enrolled 23 patients who were < 12 years of age.

    - In HAVEN 1, a statistically significant reduction in treated bleeds of 87% (95% CI: 72.3, 94.3; $p < 0.0001$) was seen in patients treated with Hemlibra prophylaxis vs. those who received no prophylaxis (annualized bleed rate: 2.9 vs. 23.3, respectively).
    - In HAVEN 2, 87% (95% CI: 66.4, 97.2) of children who received Hemlibra prophylaxis did not experience a bleeding episode that required treatment.

- Hemlibra carries a boxed warning for thrombotic microangiopathy and thromboembolism.

- Other warnings and precautions of Hemlibra include laboratory coagulation test interference.

- The most common adverse reactions (≥ 10%) with Hemlibra use were injection site reactions, headache, and arthralgia.

- The recommended dose of Hemlibra is 3 mg/kg by subcutaneous (SC) injection once weekly for the first 4 weeks, followed by 1.5 mg/kg once weekly.

    - After proper training in SC injection technique, a patient may self-inject, or the patient's caregiver may administer Hemlibra.
    - Self-administration is not recommended for children aged < 7 years old.

- The Genentech Access Solutions® program is available to help patients with access and reimbursement.

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- Genentech plans to launch Hemlibra by the end of the month. Hemlibra will be available as an injectable solution in single-dose vials in the following strengths: 30 mg/mL, 60 mg/0.4 mL, 105 mg/0.7 mL, and 150 mg/mL.