Esperoct® (antihemophilic factor [recombinant], glycopegylated-exei) – New drug approval

- On February 19, 2019, Novo Nordisk announced the FDA approval of Esperoct (antihemophilic factor [recombinant], glycopegylated-exei), for use in adults and children with hemophilia A for: on-demand treatment and control of bleeding episodes; perioperative management of bleeding; and routine prophylaxis to reduce the frequency of bleeding episodes.
    - Esperoct is not indicated for the treatment of von Willebrand disease.
- Esperoct, a glycopegylated form of recombinant anti-hemophilic factor, temporarily replaces the missing coagulation factor VIII needed for effective hemostasis in congenital hemophilia A patients.
    - The glycopegylated form of factor VIII has a longer half-life and slower clearance compared to the non-pegylated molecule.
- The efficacy of Esperoct was evaluated in five open-label studies in 254 male patients with severe hemophilia.
    - Overall, there were 1506 bleeds reported in 171 of 254 patients across the completed clinical trials. For on-demand treatment, 94.3% of bleeds required 1 to 2 injections for control of the bleeding episode and 87.3% of bleeds had an “excellent/good” response to first treatment.
    - The efficacy analysis of Esperoct in perioperative management included 45 major surgical procedures performed in 33 adolescent and adult subjects. The hemostatic effect of Esperoct was rated as “excellent” or “good” in 43 of 45 surgeries (95.6%), while the effect was rated as “moderate” in 2 surgeries (4.4%). No surgery had an outcome rated as “none” or “missing.
    - The efficacy of Esperoct in routine prophylaxis with every 4 day dosing was demonstrated in 186 adult/adolescent patients. The median annualized bleeding rate (ABR) for treated bleeds in adults and adolescents was 1.2 (interquartile range [IQR]: 0.0, 4.3).
    - The efficacy of Esperoct was also demonstrated in 68 children < 12 years receiving prophylactic treatment with Esperoct twice weekly. The prophylactic effect of Esperoct was demonstrated with a median ABR rate of 2.0 (IQR: 0.0, 2.8) for treated bleeds.
- The warnings and precautions of Esperoct include hypersensitivity reactions, neutralizing antibodies, and monitoring laboratory tests.
- The most frequently reported adverse reactions (≥ 1%) with Esperoct use were rash, redness, itching and injection site reactions.
- The recommended intravenous dosage and duration of treatment with Esperoct depend on the severity of the factor VIII deficiency, on the location and extent of bleeding, and on the patient’s clinical condition.
    - On-demand treatment/control of bleeding episodes: in adolescents/adults, 40 IU/kg body weight for minor/moderate bleeds and 50 IU/kg body weight for major bleeds; children (< 12 years), 65 IU/kg body weight for minor/moderate/major bleed.
    - Perioperative management (for minor/major surgery): in adolescents/adults, pre-operative dose of 50 IU/kg body weight; in children (< 12 years), pre-operative dose of 65 IU/kg body weight. The frequency of administration is determined by the treating physician.
    - Routine prophylaxis: in adolescents/adults, 50 IU/kg every 4 days; in children (< 12 years), 65 IU/kg twice weekly. A regimen may be individually adjusted to less or more frequent dosing based on bleeding episodes.
    - For additional dosing information, refer to the Esperoct drug label.

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- Due to third-party intellectual property agreements, Novo Nordisk will not be able to launch Esperoct before 2020. Esperoct will be available as a lyophilized powder in single-dose vials of 500, 1000, 1500, 2000 and 3000 IU per vial.