Veopoz™ (pozelimab-bbfg) – New orphan drug approval

- On August 18, 2023, the FDA announced the approval of Regeneron's Veopoz (pozelimab-bbfg), for the treatment of adult and pediatric patients 1 year of age and older with CD55-deficient protein-losing enteropathy (PLE), also known as CHAPLE disease.

- CHAPLE disease is an inherited immune disease that causes the complement system to become overactive. It is caused by mutations of the complement regulator CD55 gene, which can lead to the complement system attacking the body's own cells.

    - CHAPLE disease is an ultra-rare disease, with fewer than 100 patients diagnosed worldwide.
    - Severe thrombotic vascular occlusions can also occur among patients with CHAPLE disease, which can be life-threatening.

- Veopoz is a monoclonal antibody designed to block the activity of complement factor C5.

- The efficacy of Veopoz was established in a single-arm study where outcomes were compared to pre-treatment data in patients with active CD55-deficient PLE who had hypoalbuminemia. Ten patients ranging from 3 to 19 years of age (median of 8.5 years) were assessed for efficacy.

    - The median time for serum albumin to reach at least 3.5 g/dL was 15.5 days (95% CI: 8, 28). All 10 patients achieved normalization by week 12 and maintained serum albumin concentrations within the normal range through at least 72 weeks of treatment.
    - Five of the 10 patients received a total of 60 transfusions in the 48 weeks prior to treatment. In the 48 weeks after starting treatment, one patient received one albumin transfusion.
    - Nine of the 10 patients were hospitalized for a total of 268 days in the 48 weeks prior to treatment. In the 48 weeks after starting treatment, two patients were hospitalized for a total of 7 days.

- Veopoz carries a boxed warning for serious meningococcal infections.

- Veopoz is contraindicated in patients with unresolved *Neisseria meningitidis* infection.

- Additional warnings and precautions for Veopoz include other bacterial infections; systemic hypersensitivity reactions; and immune complex formation.

- The most common adverse reactions (in two or more patients) with Veopoz use were upper respiratory tract infection, fracture, urticaria, and alopecia.

- The recommended dose of Veopoz is as follows:

    - Day 1 (loading dose): Single 30 mg/kg dose administered by intravenous infusion after dilution.
    - Day 8 and thereafter (maintenance dosage): 10 mg/kg as a subcutaneous injection administered once weekly starting on day 8. The maintenance dosage may be increased to 12 mg/kg once weekly if there is inadequate clinical response after at least 3 weekly doses (ie, starting from week 4). The maximum maintenance dosage is 800 mg once weekly.

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- Regeneron’s launch plans for Veopoz are pending. Veopoz will be available as a 400 mg/2 mL (200 mg/mL) in a single-dose vial.