Uplizna™ (inebilizumab-cdon) – New orphan drug approval

- On June 11, 2020, the FDA announced the approval of Viela Bio's Uplizna (inebilizumab-cdon), for the treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive.

- NMOSD is a rare, severe, relapsing, neuroinflammatory autoimmune disease that can be fatal. Approximately 80% of all patients with NMOSD test positive for anti-AQP4 antibodies. Approximately 50% of patients with NMOSD have permanent visual impairment and paralysis caused by NMOSD attacks

    - Estimates vary, but NMOSD is thought to impact approximately 4,000 to 8,000 patients in the U.S.

- The precise mechanism by which inebilizumab-cdon exerts its therapeutic effects in NMOSD is unknown but is presumed to involve binding to CD19, a cell surface antigen presents on pre-B and mature B lymphocytes.

- The efficacy of Uplizna was established in a randomized, double-blind, placebo-controlled study in 213 patients with NMOSD who were anti-AQP4 antibody positive and 17 who were anti-AQP4 antibody negative. Patients received Uplizna or placebo. The primary efficacy endpoint was the time to the onset of the first adjudicated relapse on or before day 197.

    - The time to the first adjudicated relapse was significantly longer in patients treated with Uplizna compared to patients who received placebo (relative risk reduction 73%; hazard ratio [HR]: 0.272; $p < 0.0001$). In the anti-AQP4 antibody positive population there was a 77.3% relative reduction (HR: 0.227, $p < 0.0001$).
    - There was no evidence of a benefit in patients who were anti-AQP4 antibody negative.

- Uplizna is contraindicated in patients with:

    - A history of a life-threatening infusion reaction to Uplizna
    - Active hepatitis B infection
    - Active or untreated latent tuberculosis

- Warnings and precautions for Uplizna include infusion reactions, infections, reduction in immunoglobulins, and fetal risk.

- The most common adverse reactions (at least 10% of patients treated with Uplizna and greater than placebo) with Uplizna use were urinary tract infection and arthralgia.

- The recommended initial dose of Uplizna is 300 mg intravenous (IV) infusion followed 2 weeks later by a second 300 mg IV infusion. Subsequent doses (starting 6 months from the first infusion): single 300 mg IV infusion every 6 months.

    - Uplizna should be administered under the close supervision of an experienced healthcare professional with access to appropriate medical support to manage potential severe reactions such as serious infusion reactions.
    - Hepatitis B virus, quantitative serum immunoglobulins, and tuberculosis screening is required before the first dose of Uplizna.
    - Refer to the Uplizna drug label for additional dosing and administration recommendations.

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- Viela Bio plans to launch Uplizna in June 2020. Uplizna will be available as a 100 mg/10 mL solution in a single-dose vial