Vyvgart™ (efgartigimod alfa-fcab) – New orphan drug approval

- On December 17, 2021, the FDA announced the approval of argenx's Vyvgart (efgartigimod alfa-fcab), for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive.

- MG is a chronic autoimmune, neuromuscular disease that causes weakness in the skeletal muscles that worsens after periods of activity and improves after periods of rest. MG affects voluntary muscles, especially those that are responsible for controlling the eyes, face, mouth, throat, and limbs.

    - In MG, the immune system produces AChR antibodies that interfere with communication between nerves and muscles, resulting in weakness.
    - Severe attacks of weakness can cause breathing and swallowing problems that can be life-threatening

- Vyvgart is the first approval of a new class of medication. It is an antibody fragment that binds to the neonatal Fc receptor (FcRn), preventing FcRn from recycling immunoglobulin G (IgG) back into the blood. The medication causes a reduction in overall levels of IgG, including the abnormal AChR antibodies that are present in MG.

- The efficacy of Vyvgart was established in a 26-week, randomized, double-blind, placebo-controlled study in 167 gMG adult patients. Patients were randomized to either Vyvgart or placebo. The majority of patients were positive for AChR antibodies. The primary endpoint was the comparison of the percentage of Myasthenia Gravis-Specific Activities of Daily (MG-ADL) score responders during the first treatment cycle between treatment groups in the AChR-Ab positive population. MG-ADL assesses the impact of gMG on daily functions of 8 signs or symptoms that are typically affected in gMG.

    - In the AChR-Ab positive population, 67.7% and 29.7% of patients treated with Vyvgart and placebo, respectively, were MG-ADL responders (odds ratio 4.951, 95% CI: 2.213, 11.528; $p < 0.0001$).

- Warnings and precautions for Vyvgart include infections and hypersensitivity reactions.

- The most common adverse reactions (≥ 10%) with Vyvgart use were respiratory tract infections, headache, and urinary tract infection.

- The recommended dosage of Vyvgart is 10 mg/kg administered as an intravenous (IV) infusion over one hour once weekly for 4 weeks. In patients weighing 120 kg or more, the recommended dose of Vyvgart is 1200 mg (3 vials) per infusion.

    — Subsequent treatment cycles should be administered based on clinical evaluation. The safety of initiating subsequent cycles sooner than 50 days from the start of the previous treatment cycle has not been established.
    — Vyvgart should be administered via IV infusion by a healthcare professional.

- argenx launch plans for Vyvgart are pending. Vyvgart will be available as a 400 mg/20 mL single-dose vial.