Enspryng™ (satralizumab-mwge) – New orphan drug approval

- On August 17, 2020, the FDA announced the approval of Genentech's Enspryng (satralizumab-mwge), for the treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive.

- NMOSD is a rare, lifelong and debilitating autoimmune condition of the central nervous system that primarily damages the optic nerve(s) and spinal cord, causing blindness, muscle weakness and paralysis. NMOSD can affect individuals of any age, race and gender, but is most common among women in their 30s and 40s, and appears to occur at higher rates in people of African or Asian background.

    - NMOSD affects up to 15,000 people in the U.S.
    - AQP4 antibodies are detectable in the blood serum of around 70-80% of NMOSD patients.

- The precise mechanism by which Enspryng exerts therapeutic effects in NMOSD is unknown but is presumed to involve inhibition of interleukin (IL)-6-mediated signaling through binding to soluble and membrane-bound IL-6 receptors.

- The efficacy of Enspryng for the treatment of NMOSD in adult patients was established in two studies. Study 1 was a randomized, placebo-controlled trial in 64 patients without concurrent immunosuppressant therapy (IST) who were anti-AQP4 antibody positive. Study 2 was a randomized, placebo-controlled trial in 54 adult patients with concurrent IST who were anti-AQP4 antibody positive. Both studies also enrolled patients who were anti-AQP4 antibody negative. The primary efficacy endpoint for both studies was the time to the first confirmed relapse.

    - In study 1, the time to the first confirmed relapse was significantly longer in Enspryng treated patients vs. patients who received placebo (Risk reduction [RR]: 74%; hazard ratio [HR]: 0.26 [95% CI: 0.11, 0.63]; p = 0.0014).
    - In study 2, the time to the first confirmed relapse was significantly longer in Enspryng treated patients vs. patients who received placebo (RR: 78%; HR: 0.22 [95% CI: 0.06, 0.82]; p = 0.00143).
    - In both studies, there was no evidence of a benefit in the anti-AQP4 antibody negative patients.

- Enspryng is contraindicated in patients with active hepatitis B infection, active or untreated latent tuberculosis, and known hypersensitivity to Enspryng or any of the inactive ingredients.

- Warnings and precautions for Enspryng include infections, elevated liver enzymes, decreased neutrophil counts, and hypersensitivity reactions.

- The most common adverse reactions (≥ 15%) with Enspryng use were nasopharyngitis, headache, upper respiratory tract infection, gastritis, rash, arthralgia, extremity pain, fatigue, and nausea.

- The recommended loading dose of Enspryng for the first three administrations is 120 mg by subcutaneous (SC) injection at weeks 0, 2, and 4, followed by a maintenance dosage of 120 mg every 4 weeks.

    - Enspryng is intended for patient self-administration by SC injection under the guidance of a health care professional (HCP). After proper training in SC injection technique, a patient

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may self-inject Enspryng or the patient's caregiver may administer Enspryng, if the HCP determines that it is appropriate.
  - Enspryng should be administered by SC injection in the abdomen or thigh. Injection sites should be rotated with each administration. The injection should not be given into moles, scars, or areas where the skin is tender, bruised, red, hard, or not intact.

- Enspryng will have an annual cost of $190,000 for 13 doses, but the first year will cost about $220,000 for 15 doses.

- Genentech plans to launch Enspryng in two weeks. Enspryng will be available as a 120 mg/mL single-dose prefilled syringe