Soliris® (eculizumab) – New indication

- On June 27, 2019, the FDA announced the approval of Alexion Pharmaceuticals' Soliris (eculizumab), for the treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive.

- Soliris is also approved for the treatment of paroxysmal nocturnal hemoglobinuria, atypical hemolytic uremic syndrome, and generalized myasthenia gravis.

- NMOSD is a rare disorder where the body's immune system mistakenly attacks healthy cells and proteins in the body, most often in the optic nerves and spinal cord. Individuals with NMOSD typically have attacks of optic neuritis, which causes eye pain and vision loss. Individuals also can have attacks resulting in numbness, weakness, or paralysis of the arms and legs, along with loss of bladder and bowel control.

    - 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.
    - NMOSD can be associated with antibodies that bind to AQP4. Binding of the anti-AQP4 antibody appears to activate other components of the immune system, causing inflammation and damage to the central nervous system. It is estimated that 73% of all patients with NMOSD have AQP4 auto-antibodies.

- Soliris is the first FDA-approved treatment for NMOSD.

- The approval of Soliris for the new indication was based on a double-blind study in 143 patients with NMOSD who were anti-AQP4 antibody positive. Patients were randomized to receive Soliris or placebo. The primary endpoint was the time to the first adjudicated on-trial relapse.

    - The time to the first adjudicated on-trial relapse was significantly longer in Soliris-treated patients vs. placebo-treated patients (relative risk reduction 94%; hazard ratio 0.058; $p < 0.0001$).

- Soliris carries a boxed warning for serious meningococcal infections.

- The most common adverse reactions (≥ 10%) with Soliris use in patients with NMOSD were upper respiratory infection, nasopharyngitis, diarrhea, back pain, dizziness, influenza, arthralgia, pharyngitis, and contusion.

- The recommended dose of Soliris for the treatment of NMOSD is 900 mg administered intravenously weekly for the first 4 weeks; followed by 1200 mg for the fifth dose 1 week later; then 1200 mg every 2 weeks thereafter.

    - Healthcare professionals who prescribe Soliris must enroll in the Soliris REMS.
    - Refer to the Soliris drug label for dosing for all its other indications