Ultomiris® (ravulizumab-cwvz) – New indication

- On March 25, 2024, AstraZeneca announced the FDA approval of Ultomiris (ravulizumab-cwvz), for the treatment of adult patients with neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody positive.

- Ultomiris is also approved for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and generalized myasthenia gravis (gMG).

- The approval of Ultomiris for the new indication was based on an open-label study in 58 adult patients with anti-AQP4 antibody positive NMOSD. Efficacy assessments were based on a comparison of patients in the study with an external placebo control group from another study composed of a comparable population of adult patients with anti-AQP4 antibody positive NMOSD. The primary endpoint was the time to first adjudicated on-trial relapse.

    — No adjudicated on-trial relapses were observed in Ultomiris-treated patients during the treatment period, representing a statistically significant difference between Ultomiris and placebo in time to first adjudicated on-trial relapse ($p < 0.0001$). The hazard ratio for Ultomiris compared with placebo was 0.014 (95% CI: 0.000, 0.103), representing a 98.6% reduction in the risk of relapse.
    — Ultomiris-treated patients experienced similar improvement in time to first adjudicated on-trial relapse with or without concomitant treatment.

- Ultomiris carries a boxed warning for serious meningococcal infections.

- The most common adverse reactions (≥ 10%) with Ultomiris use for NMOSD were COVID-19, headache, back pain, arthralgia, and urinary tract infection.

- The recommended intravenous Ultomiris loading and maintenance dosing in adult and pediatric patients, one month of age or older weighing 5 kg or greater, with PNH or aHUS, or in adult patients with gMG or NMOSD weighing 40 kg or greater, is based on the patient's body weight, with maintenance doses administered every 4 or 8 weeks, starting 2 weeks after loading dose.

    — Refer to the Ultomiris drug label for complete dosing and administration recommendations.