Ultomiris® (ravulizumab-cwvz) – New orphan indication

- On April 28, 2022, AstraZeneca announced the FDA approval of Ultomiris (ravulizumab-cwvz), for the treatment of adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive.

- gMG is a rare, debilitating, chronic autoimmune neuromuscular disease. The estimated diagnosed prevalence of gMG in the U.S. is approximately 90,000.

- Ultomiris is also indicated for the treatment of adult and pediatric patients one month of age and older with paroxysmal nocturnal hemoglobinuria (PNH) and treatment of adult and pediatric patients one month of age and older with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy (TMA).

- The approval of Ultomiris for the new indication was based on a double-blind, randomized, placebo-controlled study in 175 patients with gMG and a positive anti-AChR antibody serologic test. Patients were randomized to either receive Ultomiris or placebo for 26 weeks. The primary efficacy endpoint was a comparison of the change from baseline between treatment groups in the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at week 26. This categorical scale assesses impact on daily function and ranges from 0 to 24, with the higher score indicating more impairment.

    — The change from baseline in the least squares mean MG-ADL total score was -3.1 for Ultomiris vs. -1.4 for placebo (treatment effect -1.6, 95% CI: -2.6, -0.7; $p < 0.001$).

- Ultomiris carries a boxed warning for serious meningococcal infections.

    — It is only available through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS).

- The most common adverse reactions (≥ 10%) with Ultomiris use in adult patients with gMG were diarrhea and upper respiratory tract infection.

- The recommended dose of Ultomiris for adult patients with gMG weighing 40 kg or greater consists of consists of a loading dose followed by maintenance dosing, administered by intravenous infusion. Starting 2 weeks after the loading dose administration, maintenance doses should begin once every 4 or 8 weeks, based on body weight.

- Refer to the Ultomiris drug label for dosing for all its other indications.