Voydeya™ (danicopan) – New orphan drug approval

- On April 1, 2024, AstraZeneca announced the FDA approval of Voydeya (danicopan), as add-on therapy to Ultomiris® (ravulizumab) or Soliris® (eculizumab) for the treatment of extravascular hemolysis (EVH) in adults with paroxysmal nocturnal hemoglobinuria (PNH).
    - Voydeya has not been shown to be effective as monotherapy and should only be prescribed as an add-on to Ultomiris or Soliris.
- PNH is a rare, progressive blood disorder characterized by red blood cell destruction within blood vessels.
    - A small subset of people living with PNH who are treated with a C5 inhibitor (eg, Ultomiris, Soliris) experience clinically significant EVH, which results in continued symptoms of anemia and may require blood transfusions.
- Voydeya is a first-in-class oral Factor D inhibitor. It works by selectively inhibiting Factor D, a complement system protein that plays a key role in the amplification of the complement system response.
- The efficacy of Voydeya was established in a randomized, double-blind, placebo-controlled study in 63 adults with PNH and clinically significant EVH. Patients were randomized to Voydeya or placebo for 12 weeks in addition to background Ultomiris or Soliris treatment. Efficacy was based on the change in hemoglobin (Hgb) level from baseline to week 12.
    - The mean change from baseline to week 12 in Hgb was 2.9 g/dL and 0.5 g/dL for Voydeya vs. placebo, respectively (difference 2.4, 95% CI: 1.7, 3.2; p = 0.0007).
- Voydeya carries a boxed warning for serious infections caused by encapsulated bacteria.
    - Voydeya is available only through a restricted program called Voydeya REMS.
- Voydeya is contraindicated for initiation in patients with unresolved serious infection caused by encapsulated bacteria, including *Neisseria meningitidis*, *Streptococcus pneumoniae*, or *Haemophilus influenzae* type B.
- Additional warnings and precautions for Voydeya include increased hepatic enzymes; monitoring of PNH manifestations after Voydeya discontinuation; and hyperlipidemia.
- The most common adverse reaction (≥ 10%) with Voydeya use was headache.
- The recommended dose of Voydeya is 150 mg three times a day administered orally.
- AstraZeneca launch plans for Voydeya are pending. Voydeya will be available as a 50 mg and 100 mg tablet.