Ultomiris® (ravulizumab-cwvz) – Expanded indication

- On June 7, 2021, Alexion announced the FDA approval of Ultomiris (ravulizumab-cwvz), for the treatment of adult and pediatric patients one month of age and older with paroxysmal nocturnal hemoglobinuria (PNH).
    - Ultomiris was previously approved for this indication in adults only.
- Ultomiris is also approved for the treatment of adults 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 expanded indication was based on an open-label study in Soliris® (eculizumab)-experienced and complement inhibitor treatment-naïve pediatric patients with PNH (N = 13).
    - Three of 5 complement inhibitor treatment-naïve patients and 6 out of 8 Soliris-experienced patients achieved hemoglobin stabilization by week 26, respectively.
    - Blood transfusion avoidance was reached for 11 out of 13 of patients during the 26-week primary evaluation period. One patient experienced breakthrough hemolysis during the extension period.
- Ultomiris carries a boxed warning for serious meningococcal infections.
- The recommended dosing in adult and pediatric patients, one month of age or older weighing 5 kg or greater, with PNH and aHUS consists of a loading dose followed by maintenance dosing, administered by intravenous infusion. The dosing is based on the patient's body weight, as shown in the table below. Starting 2 weeks after the loading dose administration, maintenance doses should begin once every 4 or 8 weeks, based on body weight.

Body weight range (kg)	Loading dose	Maintenance dose and dosing interval	
5 to less than 10	600 mg	300 mg	Every 4 weeks
10 to less than 20	600 mg	600 mg	
20 to less than 30	900 mg	2,100 mg	Every 8 weeks
30 to less than 40	1,200 mg	2,700 mg	
40 to less than 60	2,400 mg	3,000 mg	
60 to less than 100	2,700 mg	3,300 mg	
100 or greater	3,000 mg	3,600 mg	