Generic Name:
Ravulizumab
Trade Name:
Ultomiris
Company:
Alexion Pharmaceuticals
Notes:

FDA approved ravulizumab injection to adult patients with paroxysmal nocturnal hemoglobinuria (PNH), a rare and life-threatening blood disease. Ravulizumab is a long-acting complement inhibitor that prevents hemolysis.

According to FDA, prior to this approval, the only approved therapy for PNH required treatment every 2 weeks, which can be burdensome for patients and their families. Ravulizumab uses a novel formulation so patients need treatment only every 8 weeks, without compromising efficacy.

Patients with PNH are missing a certain protein that normally protects red blood cells from being destroyed by the patient?s immune system. Patients with PNH have sudden, recurring episodes in which red blood cells are prematurely destroyed. Episodes may be triggered by stresses on the body, such as infections or physical exertion. During these episodes, the following symptoms may occur: severe anemia, profound fatigue, shortness of breath, intermittent episodes of dark colored urine, kidney disease, or recurrent pain. PNH can occur at any age, although it is most often diagnosed in young adulthood.

Efficacy of ravulizumab was studied in a clinical trial of 246 patients who previously had not been treated for PNH. The patients were randomized to be treated with ravulizumab or eculizumab, the current standard of care for PNH. The results demonstrated that ravulizumab had similar results to eculizumab (noninferior): patients did not receive a transfusion and had similar incidence of hemolysis, as measured by the normalization of lactate dehydrogenase levels in patients? blood.

In addition, ravulizumab was studied in a second clinical trial of 195 patients with PNH who were clinically stable after having been treated with eculizumab for at least the previous 6 months. These patients were randomly selected to be treated with ravulizumab or to continue eculizumab.

Ravulizumab again demonstrated similar effects to eculizumab (noninferior) on the basis of several clinical measures, including hemolysis and avoidance of transfusion.

Common adverse effects were headache and upper respiratory infection. Health care providers are advised to use caution when administering ravulizumab to patients with any other systemic infection.

The prescribing information includes a boxed warning to advise health professionals and patients about the risk of life-threatening meningococcal infections and sepsis. Health care providers are advised to comply with the most current Advisory Committee on Immunization Practices recommendations for meningococcal vaccination in patients with complement deficiencies. Patients should be immunized with meningococcal vaccines at least 2 weeks prior to administering the first dose of ravulizumab, unless the risks of delaying treatment outweigh the risks of developing a meningococcal infection.

Patients and health care providers are advised that vaccination reduces, but does not eliminate, the risk of meningococcal infection. Patients should be monitored for early signs of meningococcal infections and evaluated immediately if infection is suspected.

Ravulizumab is available only through a Risk Evaluation and Mitigation Strategy (REMS) program. It must be dispensed with a patient Medication Guide that describes important information about the drug?s uses and risks.
Medication Monitor Categories:
New Drug Approvals
