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ASIA EDITION

Alexion receives early FDA approval For ULTOMIRIS

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The approval of ULTOMIRIS comes ahead of the Prescription Drug User Fee Act (PDUFA) date of February 18, 2019, set by the FDA as part of an expedited eight month review following Alexion's use of a rare disease priority review voucher

Alexion Pharmaceuticals, Inc. has announced that the U.S. Food and Drug Administration (FDA) has approved ULTOMIRIS™ (ravulizumab-cwvz), the first and only long-acting C5 complement inhibitor administered every eight weeks, for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH).

PNH is a debilitating ultra-rare blood disorder characterized by complement-mediated destruction of the red blood cells (hemolysis). It can cause a wide range of debilitating symptoms and complications, including thrombosis, which can occur throughout the body, and result in organ damage and premature death.

John Orloff, Executive Vice President and Head of Research & Development at Alexion said, "We are proud to bring ULTOMIRIS to patients suffering from this devastating disease less than a year after reporting our positive Phase 3 data. Immediate and complete C5 inhibition with ULTOMIRIS, sustained for eight weeks, can provide meaningful benefits for patients and their families. Based on the totality of our compelling data from the largest Phase 3 program ever conducted in PNH, we believe ULTOMIRIS has the potential to become the new standard of care for patients with PNH."

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Neil Horikoshi, Chief Executive Officer of the Aplastic Anemia and Myelodysplastic Syndrome (AAMDS) International Foundation said, “We applaud the FDA’s approval of ULTOMIRIS. It is important news for patients with PNH and their families. The introduction of SOLIRIS (eculizumab) eleven years ago transformed the lives of patients with PNH. But the management of this debilitating disease still requires strength and sacrifice from patients and their families. With ULTOMIRIS, patients no longer have to plan their lives around bi-weekly infusions and can look forward to just six or seven infusions a year.”

Ilene Weitz, Associate Professor at the Keck School of Medicine at the University of Southern California in Los Angeles said, “ULTOMIRIS is a compelling new therapy for patients with PNH. It has met the high bar for efficacy and safety established by SOLIRIS and has a four times longer dosing interval. I am particularly pleased by the positive data in patients transitioning from SOLIRIS to ULTOMIRIS without interruption, which is critical when you treat a devastating disease like PNH. This gives me confidence in recommending that patients switch therapy.”

This approval is based on comprehensive results from two Phase 3 studies, which were recently published in Blood.

In these studies, which included 441 patients who had either never been treated with a complement inhibitor before, or who had been stable on SOLIRIS, the efficacy of ULTOMIRIS administered every eight weeks was non-inferior to the efficacy of SOLIRIS administered every two weeks on all 11 endpoints. The safety profile of ULTOMIRIS was similar to that of SOLIRIS.

Recently presented additional data showed that ULTOMIRIS provided immediate and complete C5 inhibition that was sustained for eight weeks and that ULTOMIRIS eliminated breakthrough hemolysis associated with incomplete C5 inhibition.

The entire clinical development program for ULTOMIRIS to date represents more than 600 patient years of experience.

Regulatory authorities in the European Union (EU) and Japan have accepted and are reviewing applications for the approval of ULTOMIRIS as a treatment for adults with PNH. Alexion continues to work with the authorities to enable timely reviews.