

【Industry 2】

Revolutionizing the Standard of Care for aHUS Patients

Hsin-Yu Chen

Division of Nephrology, Kaohsiung Veterans General Hospital

Atypical hemolytic uremic syndrome (aHUS) is a rare, progressive disorder characterized by complement-mediated thrombotic microangiopathy (TMA), which, if left untreated, can result in severe organ damage and death. Ravulizumab, a next-generation long-acting C5 inhibitor, provides sustained terminal complement inhibition with a reduced dosing frequency compared to Eculizumab. This abstract consolidates findings from recent studies evaluating the safety and efficacy of Ravulizumab in aHUS patients, including those transitioning from Eculizumab.

Two-year follow-up data from Phase 3 trials demonstrated that Ravulizumab consistently maintained stable renal function and hematological parameters in both adult and pediatric cohorts. Real-world data from a global registry analysis examined 60 patients who switched from Eculizumab to Ravulizumab. The study reported no new occurrences of dialysis, kidney transplantation, or TMA events post-switch, with 20 adverse events observed in 13 patients, none of which were unexpected. Another global registry analysis focused on long-term outcomes, revealing stable estimated glomerular filtration rate (eGFR), platelet counts, and lactate dehydrogenase levels, further supporting the sustained efficacy of Ravulizumab.

Across all studies, no meningococcal infections or deaths were recorded, and the adverse events reported were consistent with the known safety profile of C5 inhibitors. The extended dosing interval of Ravulizumab improved treatment adherence and reduced the burden on patients, significantly enhancing their quality of life. These findings highlight Ravulizumab as a safe and effective therapeutic option for managing aHUS, particularly in patients transitioning from Eculizumab.

In conclusion, Ravulizumab offers robust and sustained complement inhibition, ensuring effective disease control in aHUS while providing practical advantages through reduced dosing frequency. This positions Ravulizumab as a crucial option in the evolving landscape of aHUS management.

