PNH Management in Korea: Therapeutic Options, Access and Real-World Outcomes

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Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, chronic, and potentially life-threatening hematologic disorder caused by complement-mediated hemolysis resulting from deficiency of GPI-anchored complement regulatory proteins. Over the past two decades, the treatment landscape of PNH has evolved substantially, beginning with terminal complement inhibition and expanding to proximal complement inhibitors. However, the real-world impact of these therapeutic advances may differ across countries depending on drug approval, access, and reimbursement.

This presentation first reviews the pathogenesis, clinical manifestations, and disease burden of PNH, highlighting the role of uncontrolled complement activation in intravascular hemolysis, thrombosis, organ damage, and impaired quality of life. It then summarizes globally approved PNH therapies, including terminal complement inhibitors such as eculizumab and ravulizumab, as well as proximal inhibitors including pegcetacoplan, iptacopan, and danicopan. Particular attention is given to the clinical rationale for proximal inhibition in patients with persistent anemia or ongoing extravascular hemolysis despite C5 inhibitor therapy.

The presentation also discusses the Korean treatment landscape, including currently approved agents and the evolving accessibility of complement inhibitors in Korea. Finally, Korean real-world data are reviewed, with emphasis on long-term experience with eculizumab, sustained control of intravascular hemolysis, improvement in PNH-related complications, and favorable overall survival outcomes in routine clinical practice. Real-world studies of Korean patients treated with ravulizumab are currently ongoing, and additional evidence is expected to emerge as the use of proximal complement inhibitors expands in clinical practice.

The management of PNH has entered a new era marked by multiple effective complement-targeted therapies. In Korea, expanding therapeutic access and accumulating real-world evidence are improving the outlook for patients with PNH. Ongoing efforts are needed to optimize treatment selection and improve access to novel agents that address residual anemia and extravascular hemolysis.