

Special Issue

Paroxysmal Nocturnal Hemoglobinuria: Pathophysiology and Novel Therapeutic Approaches

Message from the Guest Editor

Paroxysmal nocturnal hemoglobinuria (PNH) is a hematopoietic stem cell disorder characterized by complement-mediated intravascular hemolysis caused by the clonal expansion of hematopoietic stem cells with a mutation in a gene involved in GPI-anchor synthesis, such as *PIGA*. At this moment, the mechanism of PNH clonal expansion is still not fully resolved. The anti-C5 antibody eculizumab (Soliris®), a terminal complement inhibitor, was developed as a therapeutic for PNH hemolysis, and it not only markedly inhibited hemolysis and prevented thrombosis, but it also dramatically improved both quality of life and prognosis. However, to overcome the new challenge of manifesting extravascular hemolysis via complement C3 opsonization, various proximal complement inhibitors targeting C3, Factor D, and Factor B are being developed. This Special Issue focuses on the molecular pathogenesis of PNH and therapeutic strategies for novel anticomplement agents.

Guest Editor

Dr. Jun-Ichi Nishimura

Department of Hematology and Oncology, Osaka University Graduate School of Medicine, Suita, Japan

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4052 Basel, Switzerland
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