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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Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland
Tel: +41 61 683 77 34
ijms@mdpi.com

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The International Journal of Molecular Sciences (*IJMS*, ISSN 1422-0067) is an open access journal, which was established in 2000. The journal aims to provide a forum for scholarly research on a range of topics, including biochemistry, molecular and cell biology, molecular biophysics, molecular medicine, and all aspects of molecular research in chemistry. *IJMS* publishes both original research and review articles, and regularly publishes special issues to highlight advances at the cutting edge of research. We invite you to read recent articles published in *IJMS* and consider publishing your next paper with us.

Editor-in-Chief

Prof. Dr. Maurizio Battino
Department of Odontostomatologic and Specialized Clinical Sciences, Sez-Biochimica, Faculty of Medicine, Università Politecnica delle Marche, Via Ranieri 65, 60100 Ancona, Italy

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