(18)

血液疾病治療最新進展

The Recent Advances in the Treatment of Hematological Disease

時　　間：115 年 6 月 27 日(星期六) 13:30~17:30
地　　點：臺北榮民總醫院 致德樓第十會議室

13:30-13:40	***Opening Remarks***	蕭樑材主任 Liang-Tsai Hsiao
	座長：陳博明 醫師 (Po-Min Chen)	
13:40-14:15	慢性骨髓性白血病 CML Advancing CML Treatment with Clinical Insights on Asciminib (Virtual)	Dr. Eri Matsuki (日本)
	座長：滕傑林 醫師 (Chieh-Lin Teng)	
14:15-14:50	瀰漫性大 B 細胞淋巴瘤 DLBCL Durability and Long-Term Outcomes in DLBCL	王浩元醫師 Hao-Yuan Wang
	座長：邱宗傑 醫師 (Tzeon-Jye Chiou)	
14:50-15:25	骨髓纖維化 MF JAK Inhibitor Selection in Challenging Scenarios of Myelofibrosis (Virtual)	Prof. Claire Harrison (英國)
15:25-15:35	***Coffee Break***	
	座長：劉俊煌 醫師 (Jin-Hwang Liu)	
15:35-16:10	移植物對抗宿主疾病 GvHD From Steroid to Novel Strategies- Ruxolitinib in GvHD Management	劉耀中醫師 Yao-Chung Liu
	座長：高志平 醫師 (Jyh-Pyng Gau)	
16:10-16:45	陣發性夜間血紅素尿症 PNH Real-World Clinical Experience with Fabhalta: Redefining PNH Management in the Proximal Inhibition Era (Virtual)	Dr. Junichi Nishimura (日本)
	座長：洪英中 醫師 (Ying-Chung Hung)	
16:45-17:20	嚴重再生不良性貧血 SAA Clonal Evolution and Long-term Safety in SAA Patients Treated with EPAG: A 5-Year Follow-up Review	柯博伸醫師 Po-Shen Ko
17:20-17:30	***Coffee Break***	蕭樑材主任 Liang-Tsai Hsiao

Advancing CML treatment with clinical insights on asciminib

慢性骨髓性白血病 CML

Eri Matsuki

School of Medicine, Keio University, Japan

Despite advances in chronic myeloid leukemia (CML) therapy, unmet needs remain in frontline care, including suboptimal molecular responses, treatment intolerance, and the need for long-term disease control. Asciminib, the first STAMP (Specifically Targeting the ABL Myristoyl Pocket) inhibitor, offers a novel mechanism distinct from ATP-competitive TKIs, providing an alternative approach for patients across treatment lines.

Phase 3 frontline data from ASC4FIRST demonstrated higher major molecular response rates at Week 48 with asciminib compared with standard-of-care TKIs, alongside a favorable safety and tolerability profile. Clinical experience suggests that transition to asciminib may be considered for patients requiring optimization of safety, tolerability, or depth of response.

Mutation testing remains essential in guiding treatment decisions, particularly in the context of resistance or intolerance to ATP-binding TKIs, where asciminib's allosteric mechanism provides a complementary strategy and recommended by 2025 CML ELN guideline for multiple BCR::ABL1 mutations.

This presentation will review emerging frontline evidence, real-world clinical experience with treatment transition, and practical considerations in mutation-guided management, highlighting how asciminib may address unmet needs and support personalized CML therapy in the frontline setting.

Durability and long-term outcomes in DLBCL

瀰漫性大 B 細胞淋巴瘤 DLBCL

Hao-Yuan Wang

王浩元

Division of Hematology, Taipei Veterans General Hospital, Taipei, Taiwan, ROC

臺北榮民總醫院 血液科

Diffuse large B-cell lymphoma (DLBCL) remains a curable disease for a substantial proportion of patients; however, durability of response and long-term outcomes continue to represent critical challenges. This presentation will explore key determinants of sustained remission, including depth of response, minimal residual disease, and biological heterogeneity. We will review real-world and clinical trial evidence on long-term survival, highlighting the plateau in cure rates with standard immunochemotherapy and the unmet needs among high-risk subgroups.

Emerging therapeutic strategies—such as targeted agents, antibody-drug conjugates, and cellular therapies including CAR-T—have demonstrated promising efficacy in improving durability, particularly in relapsed or refractory settings. Among these, CAR-T represents one of several treatment options that may offer meaningful clinical benefit for selected patients, with the potential to achieve durable responses in specific clinical scenarios.

In addition, we will examine factors influencing late relapse, treatment-related toxicity, and survivorship issues, emphasizing the importance of balancing efficacy with quality of life. By integrating clinical insights with evolving treatment paradigms, this session aims to provide a comprehensive perspective on how to enhance durable responses and improve long-term outcomes for patients with DLBCL.

JAK inhibitor selection in challenging scenarios of myelofibrosis

骨髓纖維化 MF

Claire Harrison

Deputy Chief Medical Officer -Research, Data and Analytics, Guy's and St Thomas' NHS Foundation Trust UK

The therapeutic landscape for myelofibrosis (MF) has significantly evolved with the approval of multiple JAK inhibitors. However, clinical decision-making remains complex in “challenging scenarios”, such as managing severe symptom burdens, cytopenias, or transitioning between therapies.

In this presentation, I will utilize real-world hypothetical cases to navigate these complexities. A primary focus will be the enduring clinical value of Jakavi (ruxolitinib). As the most established agent with over 12 years of evidence, Jakavi remains the “gold standard” for rapid spleen volume reduction and symptom relief.

I will discuss how Jakavi’s robust long-term data—the strongest in the class—demonstrates a clear correlation between optimal dosing and improved overall survival (OS). Furthermore, I will address the safety and advantages of peritransplant Jakavi use, noting its role in achieving stable disease control before hematopoietic stem cell transplant. While newer agents offer specialized benefits for cytopenic phenotypes, Jakavi continues to serve as the essential therapeutic foundation for a broad spectrum of MF patients. Attendees will gain expert insights into balancing individual patient characteristics with the proven, disease-modifying potential of Jakavi to optimize long-term clinical outcomes.

From steroid to novel strategies- Ruxolitinib in GvHD management

移植物對抗宿主疾病 GvHD

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Graft-versus-host disease (GvHD) remains a major complication following allogeneic hematopoietic stem cell transplantation, traditionally managed with systemic corticosteroids as first-line therapy. However, steroid-refractory or steroid-dependent GvHD continues to pose significant clinical challenges, with limited efficacy and substantial toxicity. This presentation will review the evolving treatment landscape, shifting from conventional steroid-based approaches toward more targeted and mechanism-driven strategies.

Ruxolitinib, a selective JAK1/2 inhibitor, has emerged as an important treatment option in both acute and chronic GvHD, demonstrating improved response rates and symptom control in patients with inadequate response to steroids. Clinical evidence supporting its use, including key trials and real-world data, will be discussed, alongside its impact on durability of response and patient quality of life.

Beyond ruxolitinib, the session will briefly explore other novel agents and combination strategies under investigation, highlighting the trend toward personalized and steroid-sparing approaches. Practical considerations, including patient selection, timing of intervention, and management of adverse events, will also be addressed. This presentation aims to provide a comprehensive overview of how innovative therapies are reshaping GvHD management and improving long-term patient outcomes.

Real-world clinical experience with fabhalta: Redefining PNH management in the proximal inhibition era

陣發性夜間血紅素尿症 PNH

Junichi Nishimura
School of Medicine, Osaka University, Japan

This presentation explores the transformative shift in managing Paroxysmal Nocturnal Hemoglobinuria (PNH), focusing on the pivotal role of Iptacopan in redefining therapeutic success. By targeting Factor B at the proximal level of the complement cascade, this innovative mechanism provides a decisive advantage, offering comprehensive control over both intravascular and extravascular hemolysis. This shift elevates clinical expectations beyond mere transfusion independence toward the ambitious goals of hemoglobin normalization (Hb ≥12 g/dL) and profound improvements in patient quality of life.

Drawing on scientific evidence and Real-World Evidence (RWE) from Japan, I will demonstrate the rapid onset and significant magnitude of hemoglobin recovery facilitated by Iptacopan. These data underscore its ability to effectively address residual anemia and the persistent unmet needs of patients who remain symptomatic or transfusion-dependent on traditional C5 inhibitors.

Furthermore, we will examine the practical differentiation of this therapy. As a twice-daily oral regimen, Iptacopan enhances treatment stability and adherence, mitigating the risk of breakthrough hemolysis often associated with the waning effects of injectable therapies. By integrating robust clinical trial data with real-world case experiences, this session will illustrate how Iptacopan is reshaping the future of PNH care—prioritizing a patient-centric approach that minimizes medical dependency and fosters meaningful, long-term well-being in the proximal inhibition era.

Clonal evolution and long-term safety in SAA patients treated with eltrombopag: A 5-year follow-up review

嚴重再生不良性貧血 SAA

Po-Shen Ko

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Severe aplastic anemia (SAA) is a life-threatening bone marrow failure disorder with limited therapeutic options for patients ineligible for hematopoietic stem cell transplantation. Eltrombopag, a thrombopoietin receptor agonist, has demonstrated significant efficacy in improving hematologic responses when added to immunosuppressive therapy. However, concerns remain regarding long-term safety, particularly the risk of clonal evolution.

This presentation reviews 5-year follow-up data on SAA patients treated with eltrombopag, focusing on the incidence, timing, and characteristics of clonal evolution, including cytogenetic abnormalities and progression to myelodysplastic syndromes or acute leukemia. Long-term safety outcomes, including sustained response, relapse rates, and adverse events, will also be discussed.

Emerging evidence suggests that while eltrombopag contributes to durable hematologic improvement, careful longitudinal monitoring is essential to detect potential clonal changes. The overall benefit-risk profile remains favorable in appropriately selected patients, particularly in those with refractory disease.

This review aims to provide clinicians with a comprehensive understanding of long-term outcomes associated with eltrombopag use in SAA, supporting informed treatment decisions and optimized patient management strategies.