Speech Summary Book
**Speech Title:** Hematopoietic Stem Cell Transplantation Where we have come from and where do we go?

**Speaker:** James Gajewski, MD, MACP

**Affiliation:** American Society for Blood and Marrow Transplantation

**Speech Summary:**

Hematopoietic cell transplantation was developed initially as rescue following administration of high dose chemotherapy - radiation therapy treating cancer and marrow failure diseases. Originally donors were syngeneic, then HLA matched relatives and autologous. The advent of unrelated donor registries in the 1980’s, enabled unrelated transplantation - thus allowing option of allogeneic transplantation to many more patients. The rarity of donor haplotypes still meant having donor for all individuals was problematic. The late 1990’s new techniques of transplant allowed routine use of haploidentical donor, enabling almost all recipients to have a donor option. Transplant research now focuses on prevention of treatment toxicities, improved treatment of GVHD and targeted antileukemia cellular therapy. For haploidentical transplants, reduction of gvdh risk entails either the effector t cells are either removed in vivo or ex vivo, or having proliferation stunted with cytotoxic therapy. Treatment of gvdh is now moving towards targeting T cell and B cell intracellular signaling, t cell trafficking modulators, epigenetic modulation. The ability to expand t lymphocytes and NK cells, plus genetically engineer the expanded cells to target cell surface antigens have enabled the separation of gvdh from gvl and antiviral therapy. International collaboration enabled HCT to evolve from being a rare procedure to being done commonly. The IBMTR-CIBMTR, was created as an outcomes registry because single center data was not sufficient to understand the science of outcomes. This registry has provided >1000 peer-reviewed papers since 1972 with authorship from around the globe. Early in establishment of donor registries, the genetically linkages and disequilibrium within HLA necessitation necessitated world-wide unrelated donor search and harvesting. Now with cellular therapies happening around the globe, international collaboration is expanding to establishing international quality control and quality assurance inspections so that we can continue to learn from every patient treated and ensure all patients receive this therapy responsibly.
Speech Title: Past, now and future of hematopoietic stem cell transplantation (EBMT Perspectives)
Speaker: Nicolaus Kröger, MD
Affiliation: University Medical Center Hamburg-Eppendorf
Speech Summary:

For now 60 years ago, the first patients were transplanted with acute leukemia from a syngeneic graft after high dose total body irradiation. Since then, the transplant procedure has been continuously improved by better supportive care, such as infectious prophylaxis and treatment, but also improvement of HLA-typing and donor selection. Furthermore, alternative donors have become more available by establishing large unrelated donor registries around the world as well as using more umbilical cord blood and also more recently haplo-identical donors as stem cell source. Further improvement has been achieved by introducing GvHD prophylaxis with mono- or polyclonal antibodies or T-cell depletion strategies. Donor lymphocyte infusion are effective to treat or prevent relapse and use of reduced intensity conditioning has markedly decreased early transplant related mortality. All these achievements have led to a significant reduction of non-relapse mortality and a broader use of this treatment procedure is to be expected. Despite these achievements and improvement in reducing non-relapse mortality a substantial number of patients still will experience relapse. Thus, the current and future clinical efforts lie in the prevention of relapse which has become the most frequent cause of treatment failure after allogeneic stem cell transplantation. Here, novel less toxic agents, small molecules, monoclonal or bi-specific antibodies and more recently also genetic modified T-cells such as CAR-T-cells offer new possibilities within the transplant concept to reduce the risk of relapse and enhance the rate of cure.

Speech Title: How does the past predict the future of allogeneic transplantation: lessons for the field?
Speaker: Daniel Weisdorf, MD
Affiliation: University of Minnesota
Speech Summary:
Allogeneic transplantation began many years ago as replacement therapy for patients with inherited immunodeficiency disorders or marrow injury induced by radiation. While early murine experiments had demonstrated that cellular transfer could restore hematopoiesis and immunocompetence, only beginning in the late 1950s and finally in the late 1960s were successful human allogeneic transplants performed. While the original concept involved cellular transfer to restore defective hematopoiesis or immunity, it was soon extended as supportive therapy for advanced hematologic malignancies. Myeloablative conditioning transplantation for advanced acute leukemia and other disorders allowed a fraction of patients to survive in remission for some time.

Improved understanding of the immunologic components of transplantation recognized that the adoptive transfer of new, donor-derived immunocompetent cells could induce a potent antineoplastic graft versus tumor effect which was linked to, but not exclusively related, to graft-versus-host disease (GVHD). This potent immunological based graft versus tumor affect allowed extension of the curative potential of myeloablative transplantation to patients with high-risk malignancy and by the early 1980s, even to those with asymptomatic early phase chronic myelogenous leukemia.

Substantial advances in supportive care including GVHD prophylaxis, transfusion practices, plus diagnostic and therapeutic approaches to viral and fungal infections greatly facilitated expansion of the field. This allowed transplantation for many more patients in an expanding group of centers. By the late 1980s, establishment of the National Marrow Donor Program in the US and other international registries allowed many more patients who lacked matched HLA matched family donors a chance for successful transplantation. Further options, beginning in the late 1980s allowed cryopreserved umbilical cord blood (UCB) units to be used for transplantation based on their highly proliferative and immunologically naïve capacity to engraft children and smaller adults, even if not fully HLA matched. More recent recognition of novel approaches to haploidentical transplantation, particularly using post-transplant cyclophosphamide or combined multicomponent regimens with intensive GVHD prophylaxis now offer suitable allogeneic donors for nearly every patient from amongst their relatives, volunteer adult unrelated donors or UCB units.
In the mid to late 1990s, recognition of the immunologic potency of allografts to limit relapse facilitated extension of allotransplantation to older or more frail patients through the use of reduced intensity or non-myeloablative conditioning regimens. Observed experience with the hierarchy of GVL sensitive diseases facilitated transplantation for numerous hematologic malignancies in remission and recognized particular sensitivity of certain lymphoid malignancies to this powerful GVL effect.

Notably, relapse remains as the biggest challenge and the most frequent cause of posttransplant failure. Novel approaches to improve the antineoplastic potency of transplantation are the next wave of advances we can expect. These advances may come through post-transplant maintenance therapy, through antitumor vaccines or targeted therapy directed towards the tumor or even through supplemental cellular therapy. Cell therapies may include donor lymphocyte infusions, adaptively transferred natural killer (NK) cells, antigenically directed T cells (chimeric antigen or other) and other advances yet to be contemplated.

Limitations of GVHD morbidity and mortality may come through techniques to enhance regulatory T-cell (Treg) development post-transplant and techniques to induce stable tolerance without ablatting the antitumor effects may make allografting even safer and more broadly available to those most needing it; particularly older adults affected by comorbid conditions. Modern, much less morbid transplants can permit this enhanced antitumor impact and prevent relapse.

These stepwise advances in the field have developed over many years, yet are accelerating as understanding of immunologic and genetic biology accelerates in parallel. Most excitingly, more patients will be helped and more patients will be cured.
Speech Summary:

APBMT has kept growing in terms of the numbers of participating countries/regions, their clinical activities, the level of science, and the strength of our collaboration. However, our group consists of countries where the disease for which transplantation are indicated, the infrastructure for supporting transplantation, financial background, and endemic of infectious diseases vary significantly. Thus, the challenges in HSCT vary significantly among our region.

One of our most important challenges is to increase the sites to perform HSCT and the access to it. The reasons contributing to this huge supply and need discrepancies include the lack of trained personnel and the ability of the healthcare system to cover the cost of HSCT. The APBMT vision for the forthcoming years encompasses this important issue by providing emerging countries with training opportunities in HSCT and ensuring the quality of HSCT among Asia-Pacific area. APBMT will start HCT center accreditation project while harmonizing our approaches with the materials and recommendations of International Accreditation. The current FACT JACIE standards are beyond scope for 90% of centers in Asia-Pacific region, thus we are planning to commence our accreditation by step-up approach to finally reach the FACT–JACIE standards. In order to increase the opportunity of clinical studies among our regions, we need to foster the activity of APBMT transplant outcome registry. However, limited resources such as trained data managers and financial support impede the growth of our registry. To overcome this obstacle, we simplifying the report forms to and introduce EDC systems for promoting capturing of data.

These are the big challenges of APBMT, however, I remain optimistic and I believe great enthusiasm for hematopoietic stem cell transplantation and the passion of Asian peoples have undoubtedly contribute significantly to achieve this goal in the very near future.
Speech Title: Palliative care in acute healthcare settings
Speaker: Margaret O'Connor
Affiliation: Monash University

Speech Summary:
The traditional model of palliative care, has emphasized care for people facing the end of their life, mainly from cancer. Models have subsequently developed to address the needs of people dying from chronic illnesses, like heart failure; and multi-drug resistant non-communicable diseases, like tuberculosis.

The connection of palliative care expertise for people dying from acute illnesses like the failure of bone marrow transplant, has been slow to develop. There are many reasons for this, including that the clinical goal for acute illnesses like bone marrow transplant is focused on seeking a cure, right up to death.

This paper addresses the worldwide development of palliative care, highlighting the above emphases. Then the issues, challenges and ethical considerations in implementing palliative care alongside acute care are discussed, in order to promote the best care possible for those for whom treatment is ineffective.
Lunch Symposium 1~4

- Date/Time: November 2nd / 12:00-13:00
- Venue: 3F, 4F

Speech Title: Update in advanced HL treatment post ISHL
Speaker: Dr Craig H. Moskowitz
Affiliation: Sylvester Comprehensive Cancer Center, University of Miami Health System, Florida, US

Speech Summary:

The 11th International Symposium on Hodgkin Lymphoma just took place in Cologne, Germany in October. A number of clinical trials with HL patients were reported. Many of these studies combined conventional chemotherapy and targeted drugs such as the antibody-drug conjugate brentuximab vedotin (BV).

We are honored to have Dr. Craig H. Moskowitz, one of the world’s leading experts on lymphoma and physician-in-chief for the Oncology Service Line at Sylvester Comprehensive Cancer Center, as our guest speaker to present the updates post ISHL.

One highlight we especially would like to present is the 5-year follow-up of the AETHERA phase III trial:

**Study overview**

- Eligible cHL patients needed to be BV-naïve, must have received auto-HSCT before randomization and have been at high risk of relapse after auto-HSCT
- N = 329 patients randomized to receive BV (n = 165) or placebo (n =164)
- Intravenous BV (1.8 mg/kg) or placebo was administered once every three weeks for up to 16 cycles (start date 30−45 days post auto-HSCT)
- In the initial AETHERA trial, PFS by independent review was significantly improved in BV than placebo patients (HR = 0.57; 95%, CI 0.40−0.81; P = 0·0013).

**Key findings**
- Median 5-year PFS: BV: not reached versus placebo: 15.8 months

- Five-year PFS rate: BV: 59% (95% CI, 51–66) versus 41% (95% CI, 33–49) [HR =0.521; 95% CI, 0.379–0.717]

- At the three-, four- and five-year follow-up, patients receiving BV presented with a reduction in PFS events of 30%, 28% and 30%, respectively, when compared to the placebo group

- In general, significantly fewer patients in the BV arm received further anti-cancer therapy (32%, n = 53) than those in the placebo arm (54%, n = 89; P < 0.0001)

**Speech Title:** Busulfan-containing conditioning regimen for autologous stem cell transplantation in patients with multiple myeloma

**Speaker:** Je-Jung Lee

**Affiliation:** Chonnam National University Hwasun Hospital, Korea

**Speech Summary:**

High dose chemotherapy followed by autologous stem cell transplantation (ASCT) is recommended as standard consolidative therapy for transplant-eligible multiple myeloma (MM) patients. The most commonly used conditioning regimen for patients with MM is high-dose melphalan (200 mg/m²; MEL200). There are ongoing efforts to find a more effective conditioning regimen. Intravenous busulfan (BU) has been developed in 2003 and has characterized by low first pass-effect to the liver. It is expected to avoid fatal hepatotoxicity such as VOD. Korean multiple myeloma working party (KMMWP) has conducted a prospective, multicenter, phase 2 study evaluating the efficacy and toxicity of intravenous BU-MEL as a conditioning regimen for ASCT in patients with MM. A total of 99 patients with MM, enrolled between January 2013 and March 2016, received intravenous BU (9.6 mg/kg) and MEL (140 mg/m²) prior to ASCT. The overall response rate after ASCT was 94.0%, including 43.5% with a sCR/CR, 27.3% with VGPR, and 23.2% with PR. The frequent severe non-hematologic toxicity (grade 3-4) was infection (26.3%) and stomatitis (15.2%). Three patients (3.2%) developed VOD. No treatment-related mortality was observed. After median follow-up of 26.1 months, the median PFS was 27.2 months (range: 13.0-41.4) and
median OS was not reached. In this study, conditioning regimen of intravenous BU-MEL was effective and tolerable. At this meeting, I will provide the Korean data of intravenous BU-MEL and BU-Thiotepa conditioning regimens for ASCT in patients with MM.

---

### Speech Title: Cytomegalovirus infection in allogeneic hematopoietic stem cell transplantation

**Speaker:** Chieh-Lin Jerry Teng  
**Affiliation:** Division of Hematology/Medical Oncology, Taichung Veterans General Hospital, Taiwan

**Speech Summary:**

Invasive fungal infection is one of most severe complications in patients with hematological diseases. Acute leukemia, allogeneic hematopoietic stem cell transplantation (allo-HSCT), graft versus host disease (GVHD), steroid are risk factors for invasive fungal infection. Among all the pathogens of invasive fungal infection, aspergillosis is the most common one. The diagnosis of invasive aspergillosis (IA) infection is challenging. According to the evidence of IA infection, the diagnosis could be stratified into possible, probable, and proven diagnosis. Regarding the treatment, the best way to treat IA infection is to prevent it. Which patients need IA prophylaxis is debatable. Currently, patients with GVHD or previous history of IA could benefit from IA prophylaxis. When the IA infection occurs, the treatment should be diagnostic-driven. For patients with biological infection only, pre-emptive therapy should be considered. For patients who are not completely responsive to medical treatment, surgical intervention is mandatory.
Keynote Lecture 1: Microbiota in HSCT

- Date/Time: November 2nd / 13:00-13:50
- Venue: Room 201

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>Gut microbiota injury in allogeneic hematopoietic stem cell transplantation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker:</td>
<td>Yusuke SHONO</td>
</tr>
<tr>
<td>Affiliation:</td>
<td>Memorial Sloan Kettering Cancer Center</td>
</tr>
</tbody>
</table>

Speech Summary:

Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is considered to be the strongest curative immunotherapy for various malignancies (primarily, but not limited to, hematologic malignancies). However, application of allo-HSCT is limited owing to its life-threatening major complications, such as graft-versus-host disease (GVHD), relapse and infections. Recent advances in large-scale DNA sequencing technology have facilitated rapid identification of the microorganisms that make up the microbiota and evaluation of their interactions with host immunity in various diseases, including cancer. This has resulted in renewed interest regarding the role of the intestinal flora in patients with hematopoietic malignancies who have received an allo-HSCT and in whether the microbiota affects clinical outcomes, including GVHD, relapse, infections and transplant-related mortality. In this presentation, we discuss the potential role of intestinal microbiota in these major complications after allo-HSCT, summarize clinical trials evaluating the microbiota in patients who have received allo-HSCT and discuss how further studies of the microbiota could inform the development of strategies that improve outcomes of allo-HSCT.
### Nursing Symposium of GVHD issue

- **Date/Time:** November 2\textsuperscript{nd} / 13:00-15:40  
- **Venue:** Room 103

| Speech Title: Extracorporeal Photopheresis – Singapore General Hospital Experience |
| Speaker: Jessica Teo Mei Ling |
| Affiliation: Singapore General Hospital |
| Speech Summary: | Extracorporeal Photopheresis (ECP) consist of separation of Peripheral Blood Mononuclear cells (MNCs) by apheresis followed by exposing MNCs extracorporeal in a collection bag to UVA light with psoralen added into it and then reinfused to the patient.  
ECP has been used to treat patients with cutaneous T cell lymphoma; graft vs host disease and a variety of immune-mediated inflammatory diseases. Particular adverse event to look out for during ECP will be hypotension. Hence patient will be monitored closely during procedure for signs of hypotension. Hypotensive episodes are managed by temporarily stopping the procedure and administration of fluids. Transient fever has been noticed in some patients within 6 to 8 hours of reinfusion of the photoactivated MNCs. Post ECP patients are advise to avoid direct or indirect sunlight for 24 hours following exposure to psoralen. If sunlight is inevitable, patients should shield their eyes and skin by concealing exposed skin or using sunscreen. Monitoring of temperature for the next 24 hours are encouraged.  
In Singapore General Hospital (SGH), ECP was started since 2008. Till date, 22 patients have been treated with ECP. Each patient will receive 12 sessions. For the first two weeks it will be twice a week. Thereafter it will be once weekly till patient’s condition makes progress. Subsequently, physician will evaluate if there is a necessity to continue. In SGH we started ECP treatment using Cellex machine since the beginning and in May 2018 we started using UVA Pit system. Now both the systems are available for use. Physician will decide which system to use base on patient’s diagnosis. In SGH, ECP has been vastly use for patient with GVHD skin; guts and liver. Only 1 patient with GVHD lung, eye and cutaneous T cell lymphoma each. Result of ECP is very affirmative. |
### Speech Title: The Nursing for Bone Marrow Transplantation Patients with Graft-versus-host Disease

**Speaker:** Shujia Liu  
**Affiliation:** Peking University People's Hospital  
**Speech Summary:** Graft-vs-host disease (GVHD) is a multisystem disease that arises as a complication of allogeneic hematopoietic stem cell transplant. It is due to recognition of the recipient's tissues by immune cells from the donor. Acute GVHD typically presents with the triad of rash, diarrhea, and hyperbilirubinemia. I will introduce the nursing for skin, intestines and liver respectively.

### Speech Title: Graft-versus-Host Disease in Children after Hematopoietic Stem Cell Transplant: A Single-Center Experience in Taiwan

**Speaker:** Ying-Mei Liu  
**Affiliation:** Chang Gung University of Science and Technology and Linkou Chang Gung Memorial Hospital  
**Speech Summary:** Hematopoietic stem cell transplantation (HSCT) is a potentially curative therapy for many life-threatening cancers and non-malignant disorders. Each year, more than 1600 pediatric patients undergo allogeneic HSCT worldwide. Graft-versus-host disease (GvHD) is a significant cause of morbidity and mortality in pediatric patients who have undergone allogeneic HSCT. The emergence of more effective approaches for preventing and treating acute and chronic GvHD has resulted in reduced transplant mortality and relatively favorable long-term outcomes among pediatric HSCT recipients. Despite improved the survival rate for many life-threatening hematological and oncological diseases, pediatric HSCT remains a risky procedure. We conducted a single-center study of pediatric HSCT recipients and examined the outcomes related to chronic GvHD and quality of life. These outcomes were assessed after patients had received allogeneic HSCT for 1 year. Chronic GvHD was found to be the primary factor associated with poor posttransplant overall quality of life and emotional and social functioning.
Understanding the symptom experiences of chronic GvHD in children is essential to guiding assessments and interventions for limiting symptom occurrence and distress.

Speech Title: Multidisciplinary approach to GVHD patients in Korea and nurses’ challenges.
Speaker: Jin Young JUNG
Affiliation: Seoul National University Hospital
Speech Summary:
Despite improvements in prevention and treatment strategies, graft versus host disease (GVHD) remains a major concern to patients undergoing hematopoietic stem cell transplantation (HSCT) and caregivers. GVHD has complex properties; (1) It is predictable but is difficult to distinguish from other conditions. (2) High grade of GVHD leads to high mortality. (3) There is no single solution for all situations. (4) It can last a life time.
Because of its properties, GVHD needs careful assessment, diagnosis and multidisciplinary intervention.
I’ll introduce some cases of multidisciplinary approaches to GVHD patients in Korea, and talk about challenges in GVHD nursing.

Speech Title: Prevention and care of Graft-versus-host disease in haploidentical stem cell transplantations for hematological malignancies in Taiwan
Speaker: Chang, Chiao-Fang
Affiliation: National Taiwan University Hospital
Speech Summary:
Since 1983, over 7000 HSCT were performed in Taiwan, and the number increased year by year. There are eighteen HSCT centers now in Taiwan, and 400 to 500 patients received HSCT every year. Anyway, to find a suitable donor is still a time-consuming work with only 60% successful rate, so that we are devoted to haploidentical stem cell transplantation (haplo-SCT) as in
other parts of the world. There are two most noteworthy strategies of haplo-SCT, i.e. the Baltimore post-transplantation cyclophosphamide (PTCy) and the Beijing G-CSF primed bone marrow plus peripheral blood stem cells (GIAC-like, G-BM/PBSC). We aimed to compare these two approaches for hematological malignancies based on the Taiwan Blood and Marrow Transplantation Registry (TBMTR). From July to December 2017, 148 patients underwent haplo-SCT, either by PTCy (n = 61) or G-BM/PBSC (n = 87), were registered. All the PTCy-based grafts were PBSCs, while all the G-BM/PBSC received both BM and PBSC. Overall, 66% of PTCy-based group still received anti-thymoglobulin (ATG) for graft-versus-host-disease (GVHD) prophylaxis, and all recipients in the G-BM/PBSC-based group received ATG. Strategies to reduce the risk of developing GVHD are important, including nursing care from donor preparation to conditioning and GVHD prophylaxis. We share our results: Patients in the G-BM/PBSC group had a significantly higher 2-year survival rate (53% vs 35%, P=0.002) and a lower 1-year non-relapse mortality rate (17% vs 42%, P=0.020). Patients in the G-BM/PBSC group had a significantly higher incidence of grade II/IV acute GvHD (56% vs 25%, P<0.001), but the rates of grade III/IV acute GvHD (16% for the G-BM/PBSC and 13% for the PTCy, P=0.560) or extensive chronic GvHD (42% vs 21%, P=0.208) were similar.

Nursing care plays important roles in the care of HSCT patient and their family; especially in allo-HSCT, the donors need to be well prepared and cared. In addition, our responsibilities still include monitoring and evaluating GVHD and related complication, as well as safely and effectively administering a multidrug regimen to prevent or treat GVHD. Furthermore, providing patient and their family with education ensuring treatment adherence and effective self-management for expected side effects is essential, and some of them even require preparation for coping with worse quality of life resulting from GVHD and subsequent complications.

Key words:
 haploidentical stem cell transplantation (haplo-SCT), Bone marrow(BM), Peripheral blood(PB), Post-transplant cyclophosphamide(PTCy), Graft-versus-host-disease (GVHD)
### Speech Title: Nursing for GVHD patients after HCT in JAPAN

**Speaker:** Chika Yoshida, RN  
**Affiliation:** National Cancer Center Hospital  

**Speech Summary:**
More than 3,500 allogeneic hematopoietic cell transplantations are carried out annually in Japan. Approximately 1000 patients undergo transplantation from related donors, 1000 from unrelated bone marrow donors, and 1300 from cord blood donors, with an increasing number of haploidentical donors and unrelated peripheral blood stem cell donors. With the recent expanding application of allogeneic transplantation, appropriate cares to prevent complications and transplant-related mortality is important. Nurses play a major role in managing symptoms, supporting self-care and solving psychosocial problems in patients with GVHD. Regarding symptom management, nurses should explain general ideas of prevention and treatment of GVHD before starting treatment, and should make efforts to share "common language" with patients and their families. In addition, efforts should be made to alleviate physical symptoms associated with damages from conditioning regimens so that patients can prepare for upcoming GVHD symptoms. Patient GVHD symptoms should be monitored carefully and necessary care should be provided based on the appropriate risk assessment of symptom appearance in individual patients. Every time the patient have a new or worsening symptom, the care method should be discussed among the multidisciplinary team and continuous care should be provided.  
Regarding self-care support, nurses should explain self-care items necessary for transplantation treatment in advance of treatment, together with their importance and actual methods. Nurses should make sure that patients are routinely compliant with appropriate self-cares. Regarding psychosocial problems, early consultation with specialists such as psychiatrists and social workers is important.  
In the presentation, I will discuss several examples of GVHD nursing cares at all treatment stages.

### Speech Title: Graft versus Host Disease

**Speaker:** Deepa Karmegam  
**Affiliation:** Apollo Cancer Centre, Chennai, India  

**Speech Summary:**
Graft-versus-host disease (GVHD) is an immune condition that occurs after transplant procedures when immune cells from the donor attack the recipient patient host's tissues; the disease is a side effect that is common after allogeneic bone marrow transplantation. In addition to bone marrow transplant procedures, GVHD can also occur after transplantation of solid organs that may contain immune system cells such as white blood cells or from a simple blood transfusion.

Tissues from healthy donors are checked prior to bone marrow transplant to see how closely matched they are to the host's own cells using HLA typing. When there is a close match in certain genetic markers, the risk of the disease is lower. The disease can range from mild to life-threatening in severity. There are two types of GVHD: acute GVHD and chronic GVHD.

The chance of developing GVHD is around 30%-40% when the donor and recipient are related and around 60%-80% when the donor and recipient are not related. The disease can affect many different organs in the body. Graft versus host disease can affect the skin, gut and liver and needs to be treated with immunosuppressive medications like steroids and cytokine inhibitors. Patients need intensive nursing care and nutritional support. Numerous advances like post transplant cyclophosphamide and extracorporeal photopheresis have helped reduce the mortality due to GVHD. Graft versus host disease can be beneficial and provide immunotherapy against cancer cells in leukaemia and lymphoma patients.
Plenary Session 1: Topic: Post-HSCT complications

- Date/Time: November 2nd / 15:30-16:40
- Venue: Room 201

<table>
<thead>
<tr>
<th>Speech Title: Cytomegalovirus infection in allogeneic hematopoietic stem cell transplantation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Chieh-Lin Jerry Teng</td>
</tr>
<tr>
<td>Affiliation: Division of Hematology/Medical Oncology, Taichung Veterans General Hospital, Taiwan</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
</tbody>
</table>

Allogeneic hematopoietic stem cell transplantation (allo-HSCT) not only improves survival times in patients with acute myeloid leukemia and acute lymphoid leukemia, but may also be the only curative therapy for very severe aplastic anemia. Nonetheless, the morbidity and mortality that are associated with allo-HSCT limit its clinical application and efficacy. Among all the complications by the allo-HSCT, *Cytomegalovirus* (CMV) reactivation is the major infectious complication between 30 and 100 days after transplantation. The clinical entity of CMV infection is unique. Reactivation of CMV appears in 60% of seropositive allo-HSCT recipients. Without appropriate treatment, asymptomatic CMV reactivation eventually progresses to symptomatic CMV diseases, which can result in death. Although the incidence of symptomatic CMV diseases has decreased significantly because of preemptive therapy, this life-threatening complication still develops in 30% of all allo-HSCT recipients. Use of antithymoglobulin, graft versus host disease, age, and haploidentical allo-HSCT are risks for CMV viremia in allo-HSCT recipients. More evidences identify the role of CMV prophylaxis, especially in allo-HSCT recipients with certain risk factors. The most optimal prophylactic schedule for CMV viremia needs further investigation.

<table>
<thead>
<tr>
<th>Speech Title: SOS and TMA after transplantation: Korean Reports</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Hee-Je Kim</td>
</tr>
<tr>
<td>Affiliation: Seoul St. Mary’s Hematology Hospital, College of Medicine, The Catholic University of Korea, Seoul, Korea</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
</tbody>
</table>
There have been numerous studies of patients complicated with vascular endothelial injury after allogeneic blood and marrow transplantation (Allo-BMT). Allo-BMT-associated non-infectious, vascular complications, including sinusoidal obstruction syndrome (SOS), thrombotic microangiopathy (TMA), are the major life-threatening issues associated with many related factors which leads to critical multi-organ failure and high rates of transplant-related mortality.

In real clinic, SOS and TMA after BMT were major contributing causes of death, which suggests that they should be prevented and/or treated at an early stage before it develops. We investigated our experiences using the available treatment options in efforts to find a successful therapeutic protocol that can enhance our understanding for the specific group patients. Further, we have tried to point out the reliable prediction factors for TMA in adult AML patients.

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>Fungal infection</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker:</td>
<td>Artit Ungkanont, M.D.</td>
</tr>
<tr>
<td>Affiliation:</td>
<td>Faculty of Medicine Ramathibodi Hospital, Mahidol University, Bangkok Thailand</td>
</tr>
<tr>
<td>Speech Summary:</td>
<td>Fungal infection remains one of the most important causes of morbidity and mortality during hematopoietic stem cell transplantation (HSCT), with reported mortality rate ranged from 29-90%. The most important risk factor of fungal infection is prolonged neutropenia while on broad spectrum antibiotics. HSCT patients have particular risk factors, namely acute graft versus host disease requiring medium to long term use of corticosteroids, and transplantation from alternate donor. Certain genetic predisposition, such as loss of function of polymorphism in dectin-1, leads to impaired immune response to Candida infection. Transplant centers in the tropical area, where weather is more suitable for fungus growth, certainly have more burden of fungal infection comparing to those in the colder weather. Clinical courses of fungal infection depend on the fungal classes which consist of yeasts, molds or dimorphic fungi. Candida spp are the most common yeast infection, usually involve gastrointestinal tract but can disseminate through mucosal barrier breakage. Invasive aspergillosis is usually found in patients with acute leukemia during induction and may have</td>
</tr>
</tbody>
</table>
impact on transplantation later on. The practice of prophylactic antifungal therapy varies among places. Oral fluconazole has been routinely practiced. Posaconazole, found to demonstrate a survival advantage in AML patients, may have further role in HSCT in certain group of patients. However, timing of antifungal treatment must be planned to avoid interaction with drugs used during transplantation. Treatment of suspected fungal infection have shifted from empiric anti-fungal therapy to diagnostic-driven antifungal therapy. The benefit of empiric treatment has been marginal, since its initiation depended just on unresolved fever after broad spectrum antibiotic and may lead to over-treatment. Combination of diagnostic methods, such as high resolution CT scan, biomarkers such as glucan and galactomannan, together with nucleic acid testing has leaded to new decision model of treatment initiation.
Plenary Session 2: Alternative donor transplantation (2): CBT and UD-HSCT

- Date/Time: November 2nd / 15:30-16:40
- Venue: Room 102

<table>
<thead>
<tr>
<th>Speech Title: Development of cellular therapy to enhance early hematopoietic/immunological recovery after CBT</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Satoshi Takahashi</td>
</tr>
<tr>
<td>Affiliation: Institute of Medical Science, University of Tokyo</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
</tbody>
</table>

While overall survival after CBT is comparable to matched related or unrelated donor transplantation and quality of life of CB recipients are quite high because of low risk of chronic graft-versus-host disease, higher transplant related mortality especially during early phase after transplant is still observed. Delayed hematopoietic and immunological recovery, because of low cell dose and naive T cells in CB unit, are major reasons of early complications after CBT. In order to overcome the limitation of small cell dose, several techniques have been developed to expand CB-derived HSPCs ex vivo including HSPC-differentiation blockers, such as nicotinamide analog, copper chelator, inducing constitutive Notch signaling, or an aryl hydrocarbon receptor antagonist. However, all those techniques need CB cells cultivation at least 2 weeks which is potentially risk for induced transcriptional and epigenetic abnormalities, and takes higher cost. These facts led us to seek for a new strategy to overcome the cell dose barrier by using multiple CB units. In our series of mouse transplantation models utilizing a variety of mouse strains, we have tested whether multiple allogeneic HSPCs in combination can enhance hematopoietic recovery. Furthermore, using clinically relevant procedures, we successfully isolated a mixture of CD34+ HSPCs from multiple frozen CB units at one time regardless of HLA type disparities. These cells were transplantable into immune-deficient mice and contributed in a mixture to human hematopoiesis. We here show a proof that multiple allogeneic HSPCs in combination exhibit bridging effects, enabling their use not only in wider application of CBT. We believe those efforts contribute CBT as effective, safe and steady stem cell source for all patients who need allogeneic stem cell transplantation.
<table>
<thead>
<tr>
<th>Speech Title: Emerging uses of cord blood in regenerative therapies of the brain</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Dr. Joanne Kurtzberg</td>
</tr>
<tr>
<td>Affiliation: Duke University Medical Center</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
<tr>
<td>Studies in children with selective inborn errors of metabolism have shown that cord blood cells, administered intravenously after myeloablative therapy, engraft in the brain. DUOC-01, a cord blood derived cellular therapy that promotes myelination, is undergoing testing to augment standard umbilical cord blood treatment in children with leukodystrophies. These observations led us to hypothesize that cord blood cells might also have efficacy treating patients with acquired brain injuries. Clinical studies to date have been performed to demonstrate safety and efficacy of intravenous infusions of autologous cord blood in babies with hypoxic ischemic encephalopathy, young children with cerebral palsy, congenital hydrocephalus and autism, and adults with acute ischemic stroke. Further development of these therapies using allogeneic cord blood products can provide access to these therapies for all.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Speech Title: Strategic umbilical cord blood cryopreservation for clinical therapeutic applications</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Kuo-Liang Yang</td>
</tr>
<tr>
<td>Affiliation: Hualien Tzu Chi Hospital</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
<tr>
<td>Hematopoietic stem cell transplantation has been widely employed for the treatment of malignant and nonmalignant blood disorders for some time. Attempts on using hematopoietic stem cell to treat neurological diseases or solid tumors have been tried occasionally in recent years. While autologous, related or unrelated bone marrow stem cells are considered as the first prioritized source of hematopoietic therapeutic stem cells, umbilical cord blood (UCB) is also accepted as an alternative cell source for patients needing bone marrow stem cell transplantation. The advantages of UCB for hematopoietic stem cell transplantations are generally recognized as its speedy and timely availability and its less stringent requirement as far as histocompatibility between donor units and their respective recipients is the concern. Nevertheless, a number of disadvantages for UCB in stem cell transplantation are being reported, e.g.,</td>
</tr>
</tbody>
</table>
tardy in engraftment and delay course in platelet and neutrophil reconstitutions. While several factors may contribute to the above-mentioned weakness of UBC in hematopoietic stem cell transplantation, adequate total nucleated cell (TNC) count for a given recipient’s body weight is probably one of the major criteria should be addressed in terms of UCB processing and preservation.

The aim of this presentation is to focus on how to bank UCB in order to increase qualified UCB for clinical application in hematopoietic stem cell transplantation.
**Satellite Symposium 1 (Sponsored by Amgen)**
- **Date/Time:** November 2\(^{nd}\) / 16:40-17:30
- **Venue:** Room 201

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>Optimal treatment for post-transplantation relapse in multiple myeloma</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Speaker:</strong></td>
<td>Jin Seok Kim</td>
</tr>
<tr>
<td><strong>Affiliation:</strong></td>
<td>Division of Hematology, Department of Internal Medicine, Yonsei University College of Medicine, Severance hospital, 50-1 Yonsei-ro, Seodaemun-gu, Seoul,</td>
</tr>
</tbody>
</table>

**Speech Summary:**

Although overall survival (OS) has been markedly improved in transplantation eligible younger multiple myeloma (MM) patients after the introduction of bortezomib-based induction therapy and autologous stem cell transplantation (ASCT), most patients eventually experience a relapse after ASCT. Indeed, with the widely use of novel agents for patients with relapse or refractory MM, including proteasome inhibitors (PIs) such as bortezomib, ixazomib and carfilzomib, the immunomodulatory drugs (IMIDs), such as thalidomide, lenalidomide and pomalidomide, and the monoclonal antibodies, such as elotuzumab and daratumumab, OS has been markedly improved. For the selecting an appropriate regimen for relapsed MM patients after ASCT, we have to consider many factors related with patients (including patient access and socioeconomics/healthcare coverage), underlying disease and previous treatment related factors. Patients who relapse less than 12 months after ASCT are considered as high-risk group even if evaluation by FISH previously classified their disease as standard risk. Patients with aggressive relapse and patients with high risk features may need multi-agent combination therapies (triplet therapy). Because bortezomib-based regimens are usually used for induction therapy in transplantation eligible MM patients, lenalidomide-based regimens such as carfilzomib+lenalidomide-dexamethasone (Rd), ixazomib-Rd, elotuzumab-Rd or daratumumab-Rd are commonly recommended in relapsed MM patients after ASCT. Lenalidomide maintenance therapy after ASCT has been approved in many countries according to the OS benefit from phase 3 clinical trials. Therefore, different strategies should be applied for the relapsed
patients on lenalidomide maintenance after ASCT. Patients whose disease progresses during the lenalidomide maintenance after ASCT are usually treated with pomalidomide-based regimens or PI (bortezomib or carfilzomib) ± daratumumab-based regimens. Although three drug regimens including PI and IMIDs should be considered as a second line therapy after ASCT, two drug regimens also can be considered especially in the low risk patients with significant comorbidities.
Satellite Symposium 2 (Sponsored by Synmosa Biopharma Corporation)
- Date/Time: November 2\textsuperscript{nd} / 16:40-17:30
- Venue: Room 102

<table>
<thead>
<tr>
<th>Speech Title: Update 2018 on Treatment Strategy for Indolent Lymphoma</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Speaker:</strong> Mathias J. Rummel</td>
</tr>
<tr>
<td><strong>Affiliation:</strong> Department of Haematology and Oncology, Hospital of the Justus-Liebig University, Giessen, Germany</td>
</tr>
</tbody>
</table>

**Speech Summary:**
The StiL conducted several trials to optimize treatment strategies for patients (pts) with follicular/indolent lymphomas (FL, iNHL). The StiL NHL1-2003, multicenter, randomized, phase III compared Bendamustine plus Rituximab (B-R) and CHOP-R as first-line treatment in pts with indolent or mantle cell lymphoma. The study demonstrated a significantly prolonged progression-free survival (PFS) in the B-R group compared to the CHOP-R group, with a median PFS of 69 vs. 31 months, respectively. Median TTNT was significantly prolonged with B-R compared with CHOP-R (HR 0.52, p < 0.001).

In another randomized multicenter phase 3 study in first-line FL, StiL NHL7-2008 MAINTAIN, the role of Rituximab maintenance (R-maintenance) following B-R was investigated: 4 versus 2 years of R-maintenance following B-R. Rationale: R-maintenance for 2 years is part of a standard treatment approach for previously untreated FL. In this study efficacy and safety of 4 versus 2 years of R-maintenance following treatment with B-R was evaluated.

Results: A total of 612 pts with FL were enrolled. 555 pts were evaluable for response, and 497 responded to B-R induction. 350 pts were randomized to 2-years or 4-years of R-maintenance. Median PFS appeared superior with 4-years versus 2-years of R-maintenance (HR 0.64). There was no difference in OS between groups. A historical comparison for PFS between responding patients given 2 years of R-maintenance in this MAINTAIN trial and subjects from the former StiL NHL1-2003 study (B-R versus CHOP-R) who received B-R only appeared to favor R-maintenance (HR 0.78).

Conclusions: The observed HR appear to favor 2 years of R-maintenance versus observation only following induction treatment with B-R. At the time of this analysis no definitive evidence supporting the benefit of a prolonged R-maintenance for 4 years was demonstrated. Updated analysis will be
presented at the APBMT meeting. Within this presentation a review of treatment strategies will be provided including recent results of international randomized studies such as an update of the PRIMA trial, the StiL trials, the GALLIUM trial (Obinutuzumab versus Rituximab combinations) and the RELEVANCE trial (Rsquare, Lenalidomide plus Rituximab versus Rituximab-chemotherapy).
Speech Title: Acute Myeloid Leukemia: Expectations in the Near Future
Speaker: Prof Noriko Usui
Affiliation: The Jikei University School of Medicine
Speech Summary:

The AML treatment landscape has changed considerably in the past few years with the introduction of novel agents such as midostaurin and gemtuzumab ozogamicin that has significantly improved patient outcomes in the front line setting. However these improvements were mainly seen in combination with chemotherapy for patients that were eligible for intensive chemotherapy and furthermore within a select group of patients that exhibit certain biomarkers. The same cannot be said for elderly patients or patients with co morbidities that are not eligible for the traditional 7+3 chemo induction therapy. This patients have been traditionally treated using lesser intensity agents such as HMAs and low dose cytarabine with an estimated median survival of anywhere between 4-10 months. These groups of patients are also deemed not suitable for transplant due to the high mortality rates encountered during such procedures. The introduction of other newer targeted molecular drugs (etc quizartinib, gilteritinib, enasidenib, venetoclax) has brought about much needed improvement in outcomes for both the chemo eligible and ineligible patients across therapy lines. These targeted drugs work via different pathways such as the JAK-Stat, MEK/MAPK, PI3K, mutated isocitrate dehydrogenase enzymes and the BCL-2 family. They are currently being studied as either monotherapy and in combination with other drugs. In summary, we expect further changes to the current treatment paradigm as more and more therapies are being sent through clinical trials and into the clinics.
Keynote Lecture 2: Cooperation of Novel Therapy and Transplantation in Treating Hematological Malignancies

- Date/Time: November 3rd / 08:10-09:00
- Venue: Room 201

**Speech Title:** Cooperation of Novel therapy and transplantation in treating hematological malignancies

**Speaker:** Ali Bazarbachi, MD, PhD

**Affiliation:** American University of Beirut

**Speech Summary:**

Disease relapse remains the first cause of mortality of hematological malignancies after allogeneic hematopoietic stem cell transplantation (allo-HCT). The risk of recurrence is elevated in acute myeloid leukemia (AML) patients with high-risk cytogenetic or molecular abnormalities, as well as when allo-HCT is performed in patients with refractory hematological malignancies or with persistent molecular or radiological (PET-CT scan) residual disease. For high risk AML and myelodysplasia (MDS), a post transplant maintenance strategy is possible, using hypomethylating agents or tyrosine kinase inhibitors (TKI) anti-FLT3 when the target is present. For Philadelphia positive acute lymphoblastic leukemia (ALL), there is a consensus for the use of TKI anti BCR-ABL as post transplant maintenance. In multiple myeloma, maintenance lenalidomide after autologous HCT prolongs survival. In lymphoma patients, maintenance rituximab after autologous transplant is promising in follicular and mantle cell lymphoma.
Plenary Session 3: GVHD

Date/Time: November 3rd / 09:00-10:10
Venue: Room 201

Speech Title: Graft-versus-Host Disease: biological insights from preclinical and clinical studies

Speaker: He Huang

Affiliation: 1. Bone Marrow Transplantation Center, the First Affiliated Hospital, Zhejiang University School of Medicine; 2. Institute of Hematology, Zhejiang University; 3. Laboratory Of Stem Cell and Immunotherapy Engineering, Zhejiang Province

Speech Summary:

Graft versus host disease (GVHD) remains frequent and a significant obstacle to allo-HSCT. Intestinal stem cells (ISCs) and their niche Paneth cells could be primary targets in Gastrointestinal (GI) aGVHD. Innate lymphoid cells (ILCs) play key roles in the biology of GI aGVHD. Recently, multiple populations of ILCs that generate IFN-γ, IL-5 and IL-13, and IL-17 and/or IL-22 have been described. We found that IL-22+γδT17 was the core of cellular crosstalk networks in intestinal aGVHD. Intestinal γδT cells, which had a higher expression of IL-17 family and tight-junction genes in aGVHD, reduced at early phase and then increased at later stage of murine aGVHD. Intestinal IL-22+γδT17 cells kept reducing at both early and later stage in aGVHD compared with non-aGVHD group. After improving the ratio of IL-22 expression in donor γδT17 cells in transplantation, we observed greater survival in the higher IL-22 group compared with normal aGVHD group. IL-22+γδT17 could also secret GM-CSF to recruit MDSCs, which could suppress Immune activation and attenuate aGVHD. MDSCs changed in consistent with IL-22+γδT17 cells and ILC3 decreased during aGVHD.

To date, no consensus has been reached regarding the optimal salvage treatment for SR-aGVHD. We performed a novel approach to treat severe SR-aGVHD with the combination of basiliximab and etanercept. At day 28, ORR (CR+PR) to treatment was 90.8% including 75.4% CRs. The incidences of CR per organ were 100%, 73.8%, and 79.7% for skin, liver, and gut involvement, respectively. Our data suggest that the combination of basiliximab and etanercept may constitute a promising new treatment option for SR-aGVHD.

Efficacy of histone deacetylase (HDAC) inhibitors and kinase inhibitors (SYK and JAK1/2 inhibitors) in GVHD was proved by multiple publications of independent groups. Further studies in larger multicenter cohorts of patients...
are needed to identify the most effective and least toxic regimens.
Plenary Session 4: Immunotherapy and Cell therapy Non-gene Modified

Date/Time: November 3rd / 09:00-10:10
Venue: Room 102

Speech Title: Immunotherapy in Hodgkin lymphoma
Speaker: Alex F. Herrera, MD
Affiliation: City of Hope National Medical Center
Speech Summary:

Genetic alterations of the PD-L1/PD-L2 locus on chromosome 9p24.1 are a defining biological feature of classical Hodgkin lymphoma (HL). The resulting PD-L1 expression on Hodgkin Reed-Sternberg cells as well as the PD-L1 expressed in the HL microenvironment result in an ineffective host anti-tumor immune response and make HL a ripe target for PD-1 blockade. Anti-PD-1 antibody monotherapy has been effective and well-tolerated in patients with relapsed or refractory (rel/ref) HL, with the majority of patients experiencing an objective response (about 2/3 of patients) and a median duration of response of 16.6 months in the study with the longest follow-up. Based on these data, nivolumab and pembrolizumab were FDA-approved for the treatment of advanced rel/ref HL. Evidence has emerged that patients with HL benefit from continued PD-1 blockade beyond disease progression according to traditionally-defined response criteria and that the addition of or switch to chemotherapy after anti-PD-1 antibody failure can potentially re-induce clinical response. Subsequent studies have evaluated novel anti-PD-1 based combination regimens as well as the use of anti-PD-1 antibody therapy earlier in the course of a HL patient’s therapy, including first salvage therapy for rel/ref disease (e.g. nivolumab plus brentuximab vedotin) and even first line treatment (e.g. nivolumab added to AVD chemotherapy). The current role of PD-1 blockade in HL is as monotherapy in patients with advanced rel/ref disease, but the results of ongoing studies and the evolving treatment landscape in HL will determine the role of PD-1 blockade in the future.

Other novel immunotherapies (e.g. bispecific antibodies, CAR T-cells) that are currently under study in HL will also be discussed.

Speech Title: Non-gene modified cellular immunotherapy in multiple
**Speech Title:** Multiple myeloma (MM) is characterized by generalized immune dysregulation, such as functional hypogammaglobulinemia and defects in T cell immunity, natural killer (NK) cell function, and antigen-presenting capacities of dendritic cells (DCs), resulting in susceptibility to infection as well as tumor progression. Additionally, there is a rise in immune suppressor cells, such as regulatory T cells (Tregs) and myeloid-derived suppressor cells (MDSCs), in the bone marrow microenvironment. The impairment in the function of several immune cells favors the tumor escape from immune surveillance and contributes to induce myeloma cell growth and survival. Recently, immunotherapy has emerged as a promising treatment for MM, and monoclonal antibodies, vaccines, and genetically engineered T cells may represent a new era for the treatment of myeloma. DC vaccination and NK cell therapy are very safe strategies that have shown some efficacy in a subset of MM patients and may become a crucial part of MM treatment when combined with immunomodulatory drugs, immune check-point blockades, or proteasome inhibitors. Genetically engineered T cells, such as chimeric antigen receptor (CAR) T cells or T cell receptor (TCR)-engineered T cells, have also shown encouraging results, despite of worries in terms of toxicities, in recent clinical studies of patients with MM. In this presentation, I will discuss the recent progresses of cellular immunotherapeutic approaches with vaccine using DCs and NK cells in management of MM.

**Speech Title:** Autologous Haematopoietic Stem Cell Transplant as Immunotherapy for Severe Autoimmune Diseases

**Speaker:** David D Ma

**Affiliation:** Department of Haematology and BM Transplant, St Vincent's Hospital Sydney and St Vincent Clinical School, Faculty of Medical, UNSW Sydney, Australia

**Speech Summary:**
Severe autoimmune diseases (AID) remain debilitating and potentially fatal conditions in spite of recent development of biological therapies. As the safety of haematopoietic stem cell transplant (HSCT) continues to improve, it potentially offers a single treatment that may provide sustained disease control resulting in improved quality of life and survival by elimination of the autoreactive immune cell clones and the recovery of homeostatic immunity. Modern biological therapies can cause significant side-effects and can be more costly in the long term compared to the upfront, one-off cost of HSCT. Our transplant centre has consistently contributed over the last two decades to the international effort in the research field. Data from transplant registries and clinical trials has provided mounting evidence on the type of AIDs likely to benefit from HSCT and the preferred transplant conditioning regimens. Establishment of centres of excellence with well-trained staff, collaboration among medical specialties, benchmarking by independent regulatory bodies and government support are the key elements for maintainable success. As countries become more familiar with the requirements and process of HSCT, AHSCT for AID will have a place in the treatment of patients with severe and selected type of AIDs. Supporting evidence for these issues will be presented.
**TACT Joint Symposium 1: Mesenchymal Stromal Cells: Clinical Challenges and Therapeutic Opportunities**

- **Date/Time:** November 3\textsuperscript{rd} / 09:00-10:00
- **Venue:** Room 103

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>Mesenchymal Stromal Cells: Clinical Challenges and Therapeutic Opportunities.</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Speaker:</strong></td>
<td>Jacques Galipeau, MD</td>
</tr>
<tr>
<td><strong>Affiliation:</strong></td>
<td>University of Wisconsin in Madison</td>
</tr>
<tr>
<td><strong>Speech Summary:</strong></td>
<td>Mesenchymal stromal cells (MSCs) have been the subject of clinical trials for more than a generation, and the outcomes of advanced clinical trials have fallen short of expectations raised by encouraging pre-clinical animal data in a wide array of disease models. In this presentation, important biological and pharmacological disparities in murine pre-clinical research and human translational studies are highlighted, and analyses of clinical trial failures and recent successes provide a rational pathway to MSC regulatory approval and deployment for disorders with unmet medical needs.</td>
</tr>
</tbody>
</table>
Plenary Session 5: Immunotherapy and Cell therapy_gene-modified

Date/Time: November 3rd / 10:30-11:50
Venue: Room 201

Speech Title: CAR-T TREATMENT FOR REFRACTORY RELAPSED B-CELL ALL IN CHINA

Speaker: Pei-hua Lu, M.D.
Affiliation: Lu Daopei Hospital in China

Speech Summary:

Introduction:
After a long journey of development, the technology of CAR-T immunotherapy is becoming more and more mature. Up to August 2018, the registered CAR-T clinical trials worldwide indicated that USA and China are so far leading in this field. Sixteen CAR-T companies have filed IND for clinical trials in China. Since 2015 we, Lu Daopei Hospital, have collaborated with several CAR-T companies and have been doing CD19 CAR-T clinical trials for refractory and relapsed B cell ALL patients. So far we have completed more than 400 cases in our single institute with excellent results. Here we mainly select two CAR-T clinical trials to report.

Methods:
Patients’ T cells were transduced with a lentivirus vector encoding anti-CD19-CD3ζ either with a CD28 or a 4-1BB co-stimulatory domain. All patients received a conditioning regimen of IV fludarabine (25 mg/m2/day) and cyclophosphamide (250 mg/m2/day) for 3 days before a single infusion of CAR-T cells with a median dose of $1 \times 10^5$ (0.1-10x10^5) cells/kg (CAR-T cells were provided from Beijing Immunochina Medical Science & Technology Co., Ltd; Hebei Senlang Biotechnology Co., Ltd).

Results:
On day 30 evaluation after CAR-T treatment, 91.6% patients achieved complete remission (CR) or CR with incomplete count recovery (CRI), and >85% achieved minimal residual disease (MRD)-negative CR. One-year overall survival (OS) was 76.5% and relapse-free survival (RFS) was 62.6%. Grade 0-II cytokine release syndrome (CRS) incidence was 82%, and grade III-IV, 16%. Patients with high-risk features such as CNS leukemia, high leukemia burden , CML transformed, or relapsed after allogeneic hematopoietic stem cell transplantation (allo-HSCT) also were benefited from CAR -T treatment. After CAR-T treatment, majority patients underwent
subsequent allo-HSCT in a median time of 2 months. The 1-year OS/RFS of the CAR-T bridged to allo-HSCT group was better than that of the non-transplant group (OS 87.5% vs. 63.4%, p=0.013; RFS 87.4% vs. 7.5%, p=0.001). While the OS of CR patients was significantly better than that of CRi pts (100% vs. 73.4%, p=0.038), the RFS was not yet statistically significant (75% vs. 56.4%, p=0.25). The median recurrence time in the group without additional allo-HCST was 100 days. Our two year data indicated that the RFS in the MRD (-) CR group was much better the MRD (+) CR group.

Conclusion:
High CR rate was achieved from CAR-T treatment for R/R B-ALL patients even in post allo-HSCT relapsed group. CRS was manageable. Overall, RFS was superior for patients bridging to allo-HSCT after CAR-T treatment than for those receiving CAR-T- treatment only.

Speech Title: Hematopoietic stem cell based gene therapy for blood diseases.
Speaker: Alok Srivastava
Affiliation: Christian Medical College & Centre for Stem Cell Research, Vellore, India
Speech Summary:
Gene corrected autologous hematopoietic stem cell transplantation (HSCT) has been used to successfully treat patients with immune deficiency disorders for nearly two decades. A lentiviral vector based gene replacement product has recently been licensed in Europe. In recent years, through a similar approach, there has been remarkable success in the treatment of major hemoglobin disorders – a major public health problem in the Asia Pacific region. Successful gene transfer through lenti or retro viral vectors by ex-vivo transduction of autologous HSCs resulting in expression of 4-8 G/dl transgene hemoglobin have been achieved in the treatment of patients with thalassemia major and sickle cell disease. These early successes have led to the initiation of Phase 3 studies which, if successful, can lead to the registration of the candidate product. Another major genetic disorder that is a challenge to manage in developing countries is hemophilia. Adeno associated vector (AAV) based gene therapy has now been shown to be successful for this condition in Phase 1/2 trials leading to initiation of Phase 3 trials. AAV based gene therapy can be limited by pre-existing immunity in >50% of patients and may not suitable for very young children. These gene
therapies are likely to be extremely expensive given early indications of costs from currently approved products. It is important therefore that similar products be developed in an alternative model to allow access at much lower costs. We are developing a lentiviral vector mediated gene transfer to hematopoietic stem cells approach for the major hemoglobin disorders as well hemophilia A. Details are provided in abstracts of two presentations at this meeting. Pre-clinical data show significant expression of the relevant protein. The challenge is production of high quality GMP vectors for the clinical trial which is being addressed by appropriate collaborations and development of local expertise.
### Speech Title: Allogeneic Mesenchymal Stem Cells Therapy: feasibility using HLA-matched donor?

**Speaker:** Yao-Chang Chen M.D.

**Affiliation:** National Taiwan University Hospital

**Speech Summary:**

Mesenchymal Stem Cells (MSCs) are considered immunoprivileged because they express HLA-Class I but not Class II antigens. Consequently, HLA-matching is considered unnecessarily in clinical applications using allogeneic MSCs, although there have been very few studies to compare the results of matched vs mismatched major histocompatibility complex (MHC) expression. However, recent human clinical studies often showed that results using allogeneic MSCs seemed not as well as using autologous MSCs. Meanwhile, substantial evidence now exist to prove with multiple studies documenting specific cellular & humoral immunoresponse against donor follow administration of these cells. Industrial allo-MSC product failure analysis also suggested that the role of immunogenesity cannot be neglected.

We propose that the immunoprivilege property of MSCs should be re-evaluated, while the feasibility using HLA-matched allogeneic MSCs should be considered.
Speech Title: Current Role of Endothelial Progenitor Cells for Patients with End-Stage Ischemic Cardiovascular Disease

Speaker: Fan-Yen Lee

Affiliation: Kaohsiung Chang Gung Memorial Hospital, Chang Gung University, Kaohsiung, Taiwan

Speech Summary:

The population of patients with symptomatic chronic ischemic cardiac and vascular diseases is on the rise. Many of those patients remain severely symptomatic despite exhausting all conventional medical therapies. Mounting evidences suggest that microvascular insufficiency plays a noticeable role in the pathophysiology of ischemia. The science of therapeutic angiogenesis has been making much progress, and continuously evolving for over two decades.

Pre-clinical studies have provided evidence for safety and the potential therapeutic benefit of freshly isolated CD34+ cells. Clinical trials involving over several hundreds of patients have been completed providing data supporting the feasibility, safety and efficacy of CD34+ cell therapy for the treatment of refractory advanced cardiovascular disease. We will also share our experience with a prospective randomized double-blinded phase I clinical trial, using circulation-derived autologous CD34+ cells in treating patients with end-stage diffuse coronary artery disease. We define end-stage diffuse coronary artery disease as diffusely obstructive coronary artery disease, which is poorly responsive to optimal medication and also unsuitable for either percutaneous coronary artery intervention or coronary artery bypass grafting.

The goal of ischemic tissue repair appears within reach and is entering a pivotal point of clinical trial for patients with critical ischemic cardiovascular diseases.

In order to take the full advantage of this novel therapeutic strategy, the advancement of CD34+ cell based treatment for ischemic tissue repair will require an ongoing collaboration among clinicians, scientists, regulators, industries, payors and patients. This will be a new hope for patients who are disabled with their condition and have exhausted all conventional medical and surgical therapies.
### Lunch Symposium 5~8

- **Date/Time:** November 3rd/ 12:00-13:00
- **Venue:** 3F, 4F

#### Speech Title: Treatment Considerations in Relapsed and Refractory Myeloma

**Speaker:** Graham Jackson  
**Affiliation:** Freeman Hospital, The Newcastle Upon Tyne Hospitals NHS Foundation Trust

**Speech Summary:**
In this lecture we will demonstrate that treatments for multiple myeloma are improving all of the time and also discuss several important trends that are emerging.  
Firstly, continuous therapy is important and prolongs PFS and OS. Secondly the duration of therapy in the real world setting can differ from trial data and this will undoubtedly impact on the outcomes of therapy. Thirdly triplet therapies are superior to doublets and some triplets can overcome the adverse impact of high risk cytogenetics. Finally differing treatments can have differing side effect profiles and myeloma physicians need to be aware of the impact of treatment on their patients.

#### Speech Title: Deferasirox for the treatment of iron overload after allogeneic hematopoietic cell transplantation

**Speaker:** Tachibana Takayoshi, M.D., Ph.D  
**Affiliation:** Kanagawa Cancer Center

**Speech Summary:**
The aim of this study was to assess the safety and optimal dose of deferasirox for the treatment of iron overload after allogeneic hematopoietic cell transplantation (HCT). The primary endpoint was the maximum tolerated dose of deferasirox that was determined by the intrapatient dose escalation methods. A total of 16 patients with post-HCT iron overload were enrolled in the study. After excluding one case of early relapse, 15 remained evaluable. Their median age was 42 years (range 22–68). Median time from HCT to deferasirox administration was 9 months (range 6–84). Deferasirox was started at a dose of 5 mg/kg, and the dose was increased to 7.5 and 10 mg/kg every 4 weeks unless there were no grade ≥ 2 of adverse events.
Achievement rates of planned medication were 80% in 5 mg/kg (12 of 15), 73% in 7.5 mg/kg (11 of 15), and 60% in 10 mg/kg (9 of 15), respectively. The reasons for discontinuation of the drug were grade 2 of adverse events (n = 4), late relapse (n = 1), and self-cessation (n = 1). None of the patients developed grade ≥ 3 of adverse events or exacerbation of GVHD. Among 11 evaluable cases, mean value of ferritin decreased from 1560 ng/ml pre-treatment to 1285 ng/ml post-treatment. These data suggested that 10 mg/kg of deferasirox may be maximum tolerated dose when given after HCT. Our dose escalating method of deferasirox is useful to identify the optimal dosage of the drug in each patient.

Speech Title: How do novel agents impact patient outcomes in mantle cell lymphoma?

Speaker: KWONG, Yok-Lam

Affiliation: Chief of the Division of Haematology, Oncology and Bone Marrow Transplantation, Department of Medicine, University of Hong Kong

Speech Summary:

Mantle cell lymphoma (MCL) is a B-cell non-Hodgkin lymphoma with typically aggressive behavior. The genetic signature is the chromosomal translocation t(11;14)(q13;q32) resulting in overexpression of cyclin D1. While there is no standard of care for MCL, aggressive chemo-immunotherapy regimens containing rituximab and cytarabine followed by consolidation with autologous stem cell transplantation is the most utilized approach in young, fit patients and chemo-immunotherapy is most commonly used in older patients. Despite the improvement in response durations with currently available therapies, patients will inevitably relapse. In addition to improvements in immunochemotherapy, a succession of new molecular targets and corresponding drugs has revolutionized MCL therapy. The discovery of a novel agent which disrupts external signaling pathways through inhibition of Bruton's tyrosine kinase has been a particularly exciting breakthrough. The best way to sequence and combine these agents with existing regimens and how to overcome the problem of drug resistance represent new challenges in this rapidly developing field.
Speech Title: Importance of Outcome Data Sharing in Asia-Pacific Region, WBMT 5th Work Shop Report

Speaker: Yoshiko Atsuta

Affiliation: Japanese Data Center for Hematopoietic Cell Transplantation Registry Committee, APBMT

Speech Summary:

Collection and analysis of information on diseases and post-transplant courses of hematopoietic cell transplant (HCT) recipients have played important roles to the improvement of therapeutic outcome of HCT globally. The speech summarizes the discussion at the Fifth Work Shop of the Worldwide Network for Blood and Marrow Transplantation held in September 2018, in Beijing.

In 2006, the Asia-Pacific Blood and Marrow Transplantation Group (APBMT) established its registry and launched transplant activity survey from 2007. Since then, the APBMT collected data annually and 138,165 HSCT data from 624 transplant teams in 18 countries/regions were accumulated from 2007 to 2017. The data is delivered in different way from each country/region: via 1) national registry, 2) contact person from a major transplant center, or 3) each hospital/center individually. APBMT data center gathers all data and analyze it.

Regarding HCT outcome data collection, the APBMT introduced Least Minimum Data (LMD) for participating countries/regions. Data collection status in recent years will be reported. Characteristics of countries/regions which participate the APBMT are diverse in many aspects including activities of HCT, regulatory issues for medicine and medical research, economy and social infrastructure. In some countries/regions, national HCT outcome registries are active and perform important roles. The APBMT Outcome Registry basically encourage establishment of national registry in each of participating countries/regions. In this regard, the APBMT Outcome Registry perform in part as data sharing
style registry. The APBMT Data Center is currently building an electric data capture system for the LMD items based on the results of a survey in participating countries/regions. Development of this system will benefit the APBMT and some countries/regions directly by being able to provide their national data.

Speech Title: Transplant Programs in Indonesia
Report from dr. Kariadi Hospital Semarang
Speaker: Damai Santosa
Affiliation: Dr. Kariadi Hospital/ Diponegoro University
Speech Summary:
There are 3 transplant center in Indonesia, include: Dharmais National Cancer Center, Dr. Kariadi Hospital, Dr. Sutomo Hospital. There are new 10 protective room at dr. Kariadi Hospital. We have 30 nurses, which trained for bone marrow transplant services. The numbers of HSCT at dr. Kariadi Hospital in 2016-2017 are nine patients. Type of HSCT are autologous and allogeneic transplant, donor source from sibling, disease indication such as AML, MM, Lymphoma. There is not all conditioning regimen available. BMT was covered by National Health Insurance.

Speech Title: Transplant Programs in Emerging Countries: Report from MYANMAR
Speaker: Aye Aye Gyi MBBS MMedSc DrMedSc FRCP
Professor & Head, Department of Clinical Haematology
Affiliation: North Okkalapa General and Teaching Hospital
University of Medicine 2, Yangon, MYANMAR
Speech Summary:
Under minimal resources, Myanmar started the first autologous HSCT for myeloma in 2014 at the North Okkalapa General and Teaching Hospital (NOGTH), which is currently the main center while the second center is under development at the Yangon General Hospital. The government is committed to support transplant program and capacity building although health budget is insufficient to cover all the requirements. Lack of health insurance system and limited funding are the major barrier for transplant activities.
In 2016, only 5 transplant cases performed, all were autologous transplant for multiple myeloma. In 2017, allogeneic transplantation was started and clean room facilities were installed. Having only a few cases, all activities could be reported under the National Registry. There are no National Marrow Donor Program in Myanmar yet. National Blood Center of Myanmar has been trying to establish the program in near future and has started initiatives for HLA typing. Any support for the national marrow donor program would be most welcomed.

There are many limitations in promoting HSCT in Myanmar, in particular, shortage of trained person, large gap in capacity building, inadequate drug supply especially for resistant bacteria, fungi and CMV, lack of advanced laboratory facilities like HLA typing, Chimerism studies, diagnosis and monitoring of fungal and viral infections, MRD studies, etc.

However, National Collaboration was established with the centers like Singapore International Foundation and Health Authority of Singapore for transfusion workshops and transplant initiatives as well as with other centres like Siriraj Hospital, Thailand, St. Vincent’s Hospital, Sydney, Australia, Christian Medical College, Vellore and Tata Memorial Centre, Mumbai, India, etc.

The poster describing these collaborative activities in Myanmar with help from neighboring regions was awarded for the “Global Capacity Building Showcase” session at the 59th Annual Meeting of American Society of Hematology in 2017 indicating that limitations could be partly overcome by helping hands from the centers in the Asia-Pacific region despite many constraints still exist.

**Speech Title:** Transplant Program in Malaysia  
**Speaker:** Bee ping Chong  
**Affiliation:** University of Malaya  
**Speech Summary:**
Malaysia started its bone marrow/haematopoietic stem cell transplant (HSCT) program in 1987 in University of Malaya Medical Centre by the paediatric department. The first adult HSCT was done in 1993 at the same centre. There are currently 13 hospitals doing HSCT and a total of 3626 transplantation (1978 allogeneic and 1648 autologous) done. The survival has improved over the years with patients underwent transplantation from 2010 to 2015 had the best survival compared to cases done before this period. The main issues of HSCT in Malaysia are: lack of donors, inefficient laboratory supports and financial constraint. Matched unrelated donors can overcome the shortage of suitable donors, but it is limited by small local registry and expensive procurement from foreign registries. Haploidentical family donors has huge potential but collaboration with centers in the other countries is needed for skill and knowledge transfer.
Keynote Lecture 3: Cell Therapy

- Date/Time: November 3rd/ 15:35-16:25
- Venue: Room 201

<table>
<thead>
<tr>
<th>Speech Title: CAR T-cell Therapy for Lymphoma</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker: Jeremy S. Abramson, MD, MMSc</td>
</tr>
<tr>
<td>Affiliation: Massachusetts General Hospital, Harvard Medical School</td>
</tr>
<tr>
<td>Speech Summary:</td>
</tr>
</tbody>
</table>

CAR T-cells promise to transform the management of B-cell NHL. Essential components of chimeric antigen receptors (CAR) include a single chain variable region (scFv) targeting tumor antigen, a co-stimulation domain, and an intracellular signaling domain. The CAR is inserted into the patient’s T-cell, most commonly using a lentiviral or retroviral vector. Currently three major CAR T-cells are in advanced development for B-cell NHL: axicabtagene ciloleucel, tisagenlecleucel, and lisocabtagene maraleucel. All three products have an identical scFv targeting CD19, and intracellular signaling via CD3ζ. They differ, however, in their co-stimulation domains with axicabtagene employing CD28 and the other two using 4-1BB. This difference contributes to distinctions in expansion kinetics as well as timing and severity of toxicities. The products also differ in manufacturing process and dose. All three CAR T-cell products have shown remarkable clinical activity in chemotherapy-refractory DLBCL. Response rates have ranged from 52-82%, with durable remissions beyond 6 months observed in approximately 40% of subjects who have previously constituted an unmet medical need. Toxicities of these products include cytopenias, which may be prolonged, and hypogammaglobulinemia, which may require IVIG replacement. Unique toxicities of this cellular immunotherapy include a cytokine release syndrome (CRS), which may be severe and life threatening, as well as a neurologic toxicity most commonly characterized by an encephalopathy syndrome (CRES). Incidence, timing and severity of these toxicities differ across the three major CAR T-cell products, but are manageable and reversible in the large majority of cases. Ongoing areas of investigation include use of anti-CD19 CAR T-cells in additional NHL histologies, understanding mechanisms of resistance, biomarkers of response and toxicity, and optimization of the CAR construct and combination strategies.
## Speech Title: Overview of Immune Checkpoint Inhibitors in Hematologic Malignancies

**Speaker:** Chien-Chin Lin, M.D.

**Affiliation:** National Taiwan University Hospital

**Speech Summary:**

During the past few years, we have witnessed significant progress of cancer immunotherapies that activate patients’ immune systems against tumor cells. Immune-checkpoint inhibitors block the interaction between checkpoint ligands and their cognate receptors on the effector cells to augment a potent and durable tumor-killing response. The findings support the notion that cancer patients’ immunity has the capacity to react selectively to their tumors through recognition of tumor-specific antigens. Programmed death 1 (PD-1) is considered as the most important checkpoint pathways currently and the blockade has been approved by FDA for treating several solid cancers and Hodgkin lymphoma. However, the effects of PD1 blockade in various hematologic malignancies are still under investigation. Traditional treatment for hematologic malignancies include chemotherapy, radiotherapy, target therapy and allogeneic stem cell transplant. For those patients relapse or refractory to above treatment, their prognoses were poor during the past decades. In current era, immune checkpoint blockade may provide an alternative option and opportunity for them. In today’s presentation, current status and studies about immune checkpoint inhibitors in hematologic malignancies will be reviewed.
Speech Title: CAR T: a substitute or a complement to the transplant?
Speaker: Prof. Alvaro Urbano-Ispizua
Affiliation: University of Barcelona

Speech Summary:
Several American and Chinese groups have shown impressive results with Anti-CD19 directed CAR T-cell therapy in relapsed/refractory (R/R) B cell acute lymphoblastic leukemia (B-ALL), B cell non-Hodgkin lymphoma (B-NHL), and chronic lymphocytic leukemia. It was not a surprise that the U.S. Food and Drug administration (FDA) approved very fast the use of Anti CD19-CAR T in pediatric and young adults with ALL and R/R diffuse large B-NHL. CAR T cells targeting BCMA is also showing outstanding results in multiple myeloma (MM) patients previously treated with >3 lines of anti-MM regimens. Both FDA and the European Medical Agency are currently evaluating the approval of such an anti-BCMA CAR T cell. There is much enthusiasm and hope around CAR Ts. The tremendous effectiveness of the CAR T cells also raises the question of whether they will eventually substitute the hematopoietic stem cell transplantation (HSCT) or will be used as a bridge to it, or of whether they will be indicated just in case of a relapse after HSCT. To answer these questions, we need longer follow up of those patients treated with CAR T cells, and also to see how CAR Ts behave in earlier phases of these diseases. Clinical trials are already being evaluated this aspect, and results will be available in the near future. The published cost of this treatment per patient (ranging between 375,000 and 450,000 $) also raises the concern of its affordability for all European patients who might need it. Production and distribution of academic CAR T cells might be part of the solution to this economic problem. Unfortunately, less than 10% of worldwide academic CAR Ts are produced in Europe. In this presentation, the dilemma of CAR Ts vs HSCT will be discussed, together with European academic initiatives in this fascinating field of gene cell therapy.

Speech Title: Ex vivo T cell-depleted haploidentical hematopoietic cell
transplantation in children with non-malignant disorders

**Speaker:** Ho Joon Im  
**Affiliation:** Asan Medical Center Children’s Hospital, University of Ulsan College of Medicine

**Speech Summary:**
The outcomes of allogeneic hematopoietic cell transplantation (HSCT) using an HLA-haploidentical family donor have significantly improved in both T cell-depleted or T cell-replete transplants. The ex vivo techniques for removal of T cells have evolved from the selection of CD34+ hematopoietic stem cell progenitors towards the depletion of CD3+ cells and to the depletion of αβ+ T cells more recently. The most recent depletion technique targeting αβ+ T cells produces grafts containing many γδ+ lymphocytes and other effector cells including NK cells. While αβ+ T cells are known to be associated with the initiation of GVHD, γδ+ T cells can enhance immune reconstitution and are not implicated in GVHD. The αβ+ T cell depletion is the current approach applying in haploidentical HSCT at our center. More than 150 cases of haploidentical HSCT including 28 CD3-depleted transplants have been performed so far at our center. The recent emerging evidence for haploidentical HSCT has provided additional therapeutic options for pediatric patients with malignant and non-malignant diseases curable with HSCT but do not have a suitable related or unrelated donor. In this presentation, I will introduce our experience with ex vivo T cell-depleted haploidentical HCT in children and adolescents with non-malignant disorders, including bone marrow failure syndrome, primary immunodeficiency, hemophagocytic lymphohistiocytosis, and metabolic diseases.
Plenary Session 6: Alternative donor transplantation (1): Haploidentical HSCT

- Date/Time: November 4th/ 09:50-11:00
- Venue: Room 201

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>HLA-haploidentical PBSCT using posttransplant cyclophosphamide in Japan</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker:</td>
<td>Takanori Teshima, MD</td>
</tr>
<tr>
<td>Affiliation:</td>
<td>Hokkaido University</td>
</tr>
<tr>
<td>Speech Summary:</td>
<td>We conducted a series of prospective studies of HLA-haploidentical PBSCT using posttransplant cyclophosphamide (haploPBSCT; n=435). Outcome was comparable between reduced-intensity and myeloablative conditioning haploPBSCT. Survival outcomes of haploPBSCT were also comparable to those of unrelated transplantation.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Speech Title:</th>
<th>Haploidentical Transplantation In Bone Marrow Failure</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speaker:</td>
<td>Xiao-Jun Huang</td>
</tr>
<tr>
<td>Affiliation:</td>
<td>Peking University People’s Hospital</td>
</tr>
<tr>
<td>Speech Summary:</td>
<td>The recent development of haplo-HSCT in treating nonmalignant hematologic diseases, such as severe aplastic anemia (SAA), Fanconi anemia (FA), paroxysmal nocturnal hemoglobinuria (PNH) will be reviewed in this report. In 2012, Xu et al. reported on 19 consecutive SAA patients who received haplo-HSCT using Beijing protocol. In this protocol, the conditioning regimen included busulfan, cyclophosphamide and ATG. The recipients received a combination of G-CSF-primed BM and G-CSF-mobilized PBSC from haploidentical family donors and CsA, mycophenolate mofetil and short-term MTX for GVHD prophylaxis. All patients achieved 100% donor myeloid engraftment, and the OS was 64% with a median 746 days follow-up for surviving patients. Using Beijing protocol, further studies on haplo-HSCT for pediatric and adult patients with SAA as a salvage or upfront therapy were performed, respectively. And favorable outcomes were achieved. We conclude that haplo-HSCT is an effective and feasible choice for both</td>
</tr>
</tbody>
</table>
pediatric and adult patients with SAA as a salvage or even upfront therapy. 
In our center, the platform for haploidentical and unrelated donor transplantation from unmanipulated grafts in treating FA have been developed. And in this platform, the conditioning regimen includes fludarabine, dose-reduced pre-transplant CY and ATG, and without PTCY. All 5 patients including 2 haplo-HSCT recipients using this platform achieved complete donor chimerism · and 4/5 patients was alive until the date of follow-up, one haplo-HSCT recipient died of severe infection. Haplo-HSCT with Flu-containing RIC regimens may be a suitable option for FA patients without a matched related or unrelated donor. 
In summary, favorable outcomes were acquired in haplo-HSCT using Beijing protocol for nonmalignant hematological diseases, and haplo-HSCT may be a reliable strategy for patients with bone marrow failure diseases who lack a suitable matched sibling or unrelated donor.

Speech Title: Transplant outcomes of haploidentical activities, GIAC-like versus PTCy-based, for hematological malignancies in Taiwan: Results from Taiwan Blood and Marrow Transplantation Registry (TBMTR)

Speaker: Chi-Cheng Li

Affiliation: Hualien Tzu Chi Medical Center, Taiwan

Speech Summary:

Haploidentical hematopoietic stem cell transplantation represents the most difficult transplant modality in history. It also marks a milestone that human being can overcome HLA barrier to engraft successfully with controllable graft-versus-host-disease (GvHD). In the past decade, there were two well-known and introduced protocols being applied, Baltimore-designed PTCy and Beijing-designed GIAC methods. Although standing on different strategies and pathophysiology to ensure engraftment kinetics and prevention of severe GvHD, both protocols have been proved to make a big breakthrough and save thousands of lives in need of transplantation to cure various hematological disorders. Taiwan, a beautiful island located between Taiwan Strait and the Pacific Ocean, has received different influences from China and America including in the medical fields. Both PTCy-based and
GIAC-like haploidentical protocols have been introduced and practiced in Taiwan. The results have been registered to Taiwan Blood and Marrow Transplantation Registry. Updated comparison result is going to be presented to the 23rd Annual Congress of 2018 APBMT in Taipei.
Plenary Session 7: Pediatrics transplantation

Date/Time: November 4th/ 09:50-11:00
Venue: Room 102

Speech Title: Reduced-intensity conditioning Stem Cell Transplantation for pediatric patients with Primary Immunodeficiency

Speaker: Prof. Amir Ali Hamidieh
Affiliation: Tehran University of Medical Sciences

Speech Summary:
Certain types of primary immunodeficiencies (PIDs) are fatal and Allogenic Hematopoietic Stem Cell Transplantation (HSCT) is the only life-saving treatment for them, especially if therapy is instituted early, prior to onset of infections.

Reduced intensity (RIC) and myeloablative (MAC) conditioning regimens are currently being used in the treatment of patients affected by PIDs. The conditioning regimen used for HSCT in PIDs is still a controversial issue.

Full donor chimerism can be achieved with the use of MAC, but this regimen can lead to a higher risk of infections in patients with PID who suffer from comorbid complications. RIC has offered many PID patients who are ineligible for MAC regimens a chance of cure. However, the beneficial role of RIC was questioned following reports suggesting higher chance of rejection and lower symptom resolution rate in mixed chimerism settings. Despite this fact, transplant based on RIC because of its low transplant-related mortality, has been increasingly utilized in recent years.

Based on currently available international data, as pre-transplant infections in pediatric patients with PIDs increase the rate of mortality, the use of MAC regimen requires careful attention, but using less-toxic regimen with RIC seems to be highly effective and will improve manifestation of PID with either full or mixed chimerism.