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Contents

ABSTRACTS

The tables and figures attached at the time of submission have been deleted due to space limitations.

PRESIDENTIAL SYMPOSIUM

Presidential Symposium

EBMT/JACIE Perspective on "Defining Standards and Accreditation of HSCT Centers: Impact on Quality and Access"

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Hematopoietic stem cell transplantation (HSCT) has evolved into a highly specialized and life-saving therapeutic intervention for a variety of malignant and non-malignant hematological disorders. As the complexity of transplantation procedures increases, so too does the imperative to ensure standardized, high-quality care across institutions. The European Society for Blood and Marrow Transplantation (EBMT), in collaboration with the Joint Accreditation Committee of ISCT-Europe and EBMT (JACIE), has played a pivotal role in establishing and promoting comprehensive accreditation standards that encompass clinical, collection, and processing activities related to HSCT. This abstract explores the EBMT/JACIE perspective on defining and implementing these standards, assessing their impact on clinical outcomes, quality assurance, and equitable access to care.

JACIE accreditation, modeled in alignment with international best practices, has emerged as a benchmark for excellence in HSCT service delivery. Through a rigorous process of peer-reviewed audits, continual professional development, and data-driven quality improvement, the program ensures adherence to evidence-based protocols, enhancing patient safety and treatment outcomes. Numerous studies, including large-scale registry analyses conducted by EBMT, have demonstrated that JACIE-accredited centers consistently achieve better overall survival, lower transplant-related mortality, and improved management of complications compared to non-accredited centers. These outcomes underscore the critical role that structured quality frameworks play in driving superior healthcare delivery in transplantation.

Beyond clinical metrics, the EBMT/JACIE initiative has also influenced the organizational culture of transplant centers, promoting multidisciplinary collaboration, outcome transparency, and accountability. Furthermore, JACIE has served as a catalyst for the harmonization of practices across Europe and beyond, facilitating cross-border referrals, joint clinical trials, and mutual recognition of care standards. However, challenges remain in ensuring that smaller or resource-constrained centers can achieve and maintain accreditation. There is a growing

recognition of the need to balance stringent quality standards with inclusivity, particularly in regions with limited infrastructure or workforce capacity.
The EBMT/JACIE perspective emphasizes that while accreditation is a key driver of quality, its broader success depends on adaptability, stakeholder engagement, and continuous support for capacity building. Efforts are ongoing to simplify compliance pathways, provide tailored support for under-resourced centers, and incorporate patient-centered measures into the accreditation model. As the landscape of HSCT continues to evolve with novel cellular therapies and personalized medicine, the EBMT and JACIE remain committed to refining accreditation standards to ensure they remain fit-for-purpose, equitable, and globally relevant. In conclusion, the EBMT/JACIE framework represents a cornerstone in the pursuit of excellence in HSCT, demonstrating that systematic standardization not only improves quality but also has the potential to expand access and equity in life-saving transplantation services.

Presidential Symposium

Defining standards and accreditation of HSCT centers – Impact on quality and access

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Accreditation by the Joint Accreditation Committee of ISCT-Europe and EBMT (JACIE) in conjunction with the Foundation for the Accreditation of Cellular Therapy (FACT) has become a cornerstone in standardizing and improving the quality of care in bone marrow transplantation (BMT) units. These accreditation systems provide a rigorous framework encompassing clinical programs, collection facilities, and processing laboratories. The standards emphasize personnel qualifications, the implementation of quality management systems (QMS), and systematic outcome measurement.

A key benefit of JACIE-FACT accreditation lies in its focus on defining, collecting, and benchmarking outcomes, such as overall survival (OS), disease-free survival (DFS), and transplant-related mortality (TRM). Participation in centralized registries like the EBMT allows accredited centers to monitor performance indicators and address deviations proactively. Published evidence supports that accredited centers often demonstrate superior clinical outcomes and more robust safety practices compared to non-accredited counterparts.

Despite its benefits, the accreditation process presents challenges including resource demands, documentation burden, and the need for cultural and administrative change. However, these efforts foster a culture of continuous quality improvement, where mortality reviews, internal audits, and patient safety become institutional priorities.

As cellular therapy advances globally, expanding accreditation to low- and middle-income countries and incorporating novel tools such as artificial intelligence and patient-reported outcomes will be crucial. JACIE-FACT accreditation not only ensures compliance with best practices but also cultivates a learning health system focused on transparency, accountability, and excellence in patient care.

Presidential Symposium

Defining standards and accreditation of haematopoietic stem cell transplant (HSCT) centres – Impact on quality and access: the Asia-Pacific perspective

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President of the APBMT

A major limitation of haematopoietic stem cell transplantation (HSCT) services in many parts of the world – including some regions of Europe and North America, is the low HSCT density / million population. Though there could be many contributing factors, one of them is the lack of suitable large multispecialty hospitals capable of hosting such services and / or multi-physician teams to establish and maintain such HSCT centres. An alternative model has evolved in India over the last decade. Newly trained haematologists initiate haematology services within service hospitals of 100-200 bed strength with limited in-house multi-speciality care in smaller towns in the country and within 1-2 years of doing progress to adding HSCT to their services. These single transplant-physician HSCT services are performing 10-50 HSCTs / year, with other medical / nursing health care professional in the team but no other physician trained in HSCT. While there can be many limitations of such practices, it has helped many patients in India get access to HSCT nearer home who would otherwise never be able to travel to the larger HSCT centres in the big cities. Over a period of time, such services grow to add more HSCT trained physicians. Very significantly, the cost of HSCT including from a range of donors for allogeneic HSCT are much lower than in the bigger hospitals in India.

Once established, as best as possible within the limitations of their health care system, another challenge for HSCT services is to obtain formal accreditation as per established international norms – JACIE /FACT. The reality therefore is that <5% of HSCT centres in most low-and-middle income countries (LMIC) in Asia-Pacific, Latin America and Africa, and many in even some high-income countries (HIC) are not internationally accredited. While the value of such accreditation is the enhancement of quality of care, what happens when more than half the world is unable to comply with such standards? The alternatives include alignment with

national accreditation standards for hospital services or just follow institutional norms.

The critical issue is to understand the impact on access and outcomes of HSCT services in different models of care and under different types of standardization of processes with or without formal accreditation. Such data could be extracted from pooled outcomes reports from HSCT centres – with and without formal accreditation, submitted to large outcomes registries such as the CIBMTR and EBMT registries, this should be feasible. The same can also be attempted within the less extensive data on outcomes in the APBMT and LABMT registries. From such analysis, an alternate model for access, standardization and even formal accreditation could arise.

The current model for establishing HSCT services and their accreditation process and standards need review to recognize and evolve other models within the realities of existing health care systems around the world. One size never fits all and it is the same for HSCT services.

PLENARY SESSION

Plenary Session -Evolution of Conditioning

Antibody-drug conjugates targeting CD45 plus Janus kinase inhibitors effectively condition for allogeneic hematopoietic stem cell transplantation

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Allogeneic hematopoietic stem cell transplantation (allo-HSCT) holds great potential for curing numerous malignant and non-malignant hematologic diseases. However, conventional chemotherapy- and irradiation-based transplant conditioning regimens impose severe toxicity risks to patients, barring their access to this lifesaving therapy. Even antibody-drug conjugates (ADC) carry significant toxicity risks due to exposure to the extremely toxic compounds used as ADC payloads. Non-malignant diseases provide the ideal application for minimally toxic HSCT conditioning since the antitumor benefit and full donor chimerism enabled by high-intensity cytotoxic regimens are not necessary for cure. We hypothesized that naked antibody-based conditioning can achieve the therapeutic benefits of allo-HSCT for classical hematologic diseases while obviating exposure to cytotoxic agents. By combining stem cell niche depletion using anti-CD47 plus anti-CD117 (c-Kit) with immunosuppression with Janus kinase 1/2 (JAK1/2) inhibitors, we achieved stable multilineage engraftment in fully MHC-mismatched murine allo-HSCT. The near complete donor chimerism among granulocytes (95-99%) enabled correction of phagocyte oxidase function in the gp91phox$^{-/-}$ model of chronic granulomatous disease. In summary, the novel combination of CD47 and c-Kit antibodies with JAK1/2 inhibition permits HSCT across immunological barriers with robust conversion to donor hematopoiesis and, most notably, requires no exposure whatsoever to radiation, chemotherapy, or toxic ADC payloads. Recently, we have developed novel small molecule VLA-4 inhibitors that synergize with plerixafor or the higher-affinity CXCR4 antagonist motixafortide to mobilize stem cells in mouse and macaque models. We will present data demonstrating that the Townes SCD model is highly amenable to PBSC harvest for gene editing and transplantation studies, including integration with our ongoing studies into minimally toxic, antibody-based transplant conditioning.

Plenary Session -Artificial Intelligence in SCT

Hematopoietic Stem Cell Transplantation: Risk Stratification, Machine Learning, and Models

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Hematopoietic stem cell transplantation (HSCT) has shifted from a uniform approach to precision medicine, where personalized interventions optimize outcomes. This presentation highlights risk stratification and machine learning (ML) models as key drivers of this evolution. Traditional tools like IPSS-R (MDS) or ELN (AML) use limited variables (disease stage, age) with moderate accuracy (AUC ~0.6–0.7) but miss complex interactions between genetics, donor factors, and comorbidities. Precision stratification integrates multi-dimensional data—genomic mutations (FLT3, NPM1), minimal residual disease (MRD), donor HLA matching—via ML models (random forests, neural networks). These models outperform traditional tools (AUC ~0.8–0.85), predicting relapse, graft-versus-host disease (GVHD), and survival more accurately. Clinically, ML guides donor selection, adjusts conditioning intensity (reduced for low-risk patients to lower toxicity), and targets post-transplant MRD monitoring. Challenges include data standardization and model interpretability. Future steps involve global collaboration (e.g., APBMT registries), real-time data integration, and user-friendly tools. ML-driven precision will redefine HSCT, enhancing safety and efficacy through personalized care.

SESSION 1

DONOR SELECTION / ALTERNATIVE DONOR SCT

Session 1-Donor Selection/Alternative Donor SCT

Allo-SCT in the elderly - Is there an upper limit?

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Allogeneic stem cell transplantation (allo-SCT) remains the only potentially curative option for many hematologic malignancies. Given that the median age at diagnosis for these malignancies is approximately 70 years, the role of transplantation in older adults is of increasing relevance. However, most patients over the age of 70—particularly those with comorbidities—are not offered allo-SCT due to concerns about increased treatment-related mortality (TRM) and suboptimal outcomes. As a result, only about 10% of all allogeneic transplants reported to national and international registries are performed in this age group, reflecting significant underutilization of a potentially life-saving therapy.

Over the past two decades, advances in transplant strategies have improved safety and feasibility in older adults. The adoption of reduced-intensity conditioning (RIC) regimens has significantly lowered TRM. Furthermore, the incorporation of post-transplant cyclophosphamide (PTCy)-based graft-versus-host disease (GVHD) prophylaxis has revolutionized GVHD prevention, resulting in substantial reductions in both acute and chronic GVHD. These developments have collectively broadened the candidacy for allo-SCT among elderly patients.

This presentation will provide a historical overview of allo-SCT in the elderly, with a focus on how transplant regimens, GVHD prophylaxis, and supportive care strategies have evolved. The presenter will also share institutional data from a cohort of patients aged 70 years and older who underwent allo-SCT using Melphalan based RIC regimens and PTCy-based GVHD prophylaxis. These data will highlight that, with careful patient selection and current transplant strategies, allo-SCT can be safely and effectively extended to older patients with hematologic malignancies, challenging the traditional age-based limitations.

Session 1-Donor Selection/Alternative Donor SCT

Optimizing Single Cord Blood Transplantation: Insights and Progress from Japan

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Umbilical cord blood transplantation (UCBT) has played an important role in hematopoietic stem cell transplantation in Japan, with continuous improvements over the past two decades. This presentation provides an overview of recent advances in single- unit cord blood transplantation (sUCBT) in Japan and highlights similarities and differences compared to practices in the United States and Europe.

In Japan, the utilization rate of cord blood is remarkably high, with approximately 75% of units used within one year of release. Advances in unit selection criteria, conditioning regimens, and supportive care have significantly improved early survival and engraftment rates, resulting in reduced non-relapse mortality (NRM) within 100 days post-transplant. Notably, the introduction of the FluBu12.8Mel80 conditioning regimen has achieved better disease control and overall survival in patients with non-remission myeloid malignancies, outperforming conventional myeloablative approaches.

Comparative studies, including large-scale registry analyses and international collaborations, have shown that outcomes for sUCBT in Japan are comparable to or even better than those in Europe. Additionally, when compared to other alternative donor sources such as PTCy-haploidentical transplantation, UCBT demonstrates a favorable relapse profile while maintaining similar overall and relapse-free survival rates.

Interestingly, mild acute GVHD appears to confer survival benefits uniquely in UCBT cohorts, especially among Japanese patients.

Despite minor differences in transplant practices and drug availability, the fundamental principles of cord blood selection and conditioning are largely aligned internationally, supporting global sharing of strategies and outcomes. In conclusion, with appropriate graft selection and optimized regimens, UCBT remains a strong option for patients lacking matched donors, particularly those with high-risk hematologic malignancies.

Ongoing collaborative research is essential to further refine these strategies and fully realize the potential of cord blood as a valuable stem cell source worldwide.

SESSION 2

PEDIATRIC SCT
(non-malignancy)

Session 2 -Pediatric SCT(non-malignancy)

Hematopoietic stem cell transplantation for severe aplastic anemia

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Hematopoietic stem cell transplantation (HSCT) using a matched related donor (MRD) is considered a curative therapy for severe aplastic anemia (SAA). Cyclophosphamide-based conditioning combined with anti-thymocyte globulin (ATG) is generally regarded as the optimal regimen for HSCT with an MRD. However, outcomes in patients over 40 years of age have been less favorable, making the best first-line treatment option debatable. This has often led to the selection of immunosuppressive therapy (IST) over HSCT with an MRD.

For patients without a suitable MRD, IST—typically consisting of ATG and cyclosporine (CSA)—remains the standard first-line treatment. Nonetheless, with advancements in transplantation techniques, such as the incorporation of fludarabine and/or low-dose total body irradiation in conditioning regimens to reduce graft failure, promising results have emerged for HSCT using matched unrelated donors (MUD), particularly in pediatric SAA cases. These outcomes have been comparable to those achieved with MRD transplants.

The donor pool has expanded further, and recent studies have reported encouraging results for HSCT using haploidentical donors in SAA patients lacking both MRD and MUD options.

To improve HSCT outcomes for SAA, several factors must be optimized: conditioning regimens, ATG administration, donor selection, HLA matching, stem cell source, graft-versus-host disease (GVHD) prophylaxis, and supportive care. Additionally, careful consideration of short- and long-term complications—including abnormal immune reconstitution, nonmalignant organ or tissue dysfunction, delayed infections, and secondary malignancies—is essential.

Session 2 -Pediatric SCT(non-malignancy)

Challenging the Odds: Hematopoietic Stem Cell Transplantation Outcomes in Pediatric Inherited Bone Marrow Failure Syndromes

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Background: Inherited bone marrow failure syndromes (IBMFS), such as Fanconi anemia (FA), dyskeratosis congenita (DKC), and Diamond-Blackfan anemia (DBA), are rare genetic disorders characterized by defective hematopoiesis and pose significant challenges in pediatric hematology due to their progressive bone marrow failure and predisposition to malignancies. Hematopoietic stem cell transplantation (HSCT) remains the only curative option for these patients, yet its efficacy and complications in this population require detailed evaluation.

Methods: A retrospective analysis was conducted on 66 pediatric patients undergoing HSCT at the Children's Medical Center in Tehran, Iran, from January 2017 to January 2024. In all patients except those with Fanconi anemia, non-total-body irradiation (non-TBI) myeloablative conditioning (MAC) based on intravenous (IV) busulfan was employed. For Fanconi anemia patients, from 2017-2020, we used non-myeloablative conditioning consisting of oral Busulfan and cyclophosphamide with or without anti-thymocyte globulin (ATG). After 2020, non-myeloablative conditioning based on fludarabine was utilized.

Results: The study cohort comprised 56.7% male patients, with a median age at transplantation of 7.08 years [range: 1–17]. Fanconi anemia accounted for 79.1% of the study population. Grafts were primarily sourced from peripheral blood (76.1%). Donors were predominantly other related donors (34.3%), followed by sibling donors (31.3%). Fully matched transplants constituted 83.6% of cases.

The median number of infused mononuclear cells (MNC), CD34+, and CD3+ were 8.00×10^8, 4.95×10^6, and 316×10^6 CD3+ cells/kg. Neutrophil and platelet engraftment occurred at median times of 12 and 14 days, respectively. Primary engraftment was achieved in all patients except one.

Post-transplant complications included cytomegalovirus (CMV) infection in 36 patients (53.7%), acute graft-versus-host disease (GvHD) in 65.7% (with 29.8% experiencing grade III or higher), and hemorrhagic cystitis in 34.3%. During follow up autoimmune complications and chronic GvHD occurred in 1.5% and 31.4% of patients, respectively. Stable mixed chimerism was observed in 6% of

patients. Secondary graft failure requiring retransplantation affected five patients (7.5%).
The 1-year overall survival (OS) was 90.5%, and the 2-year OS was 81.6%, with infections and GvHD as the primary causes of death.
Conclusion: HSCT offers a viable curative approach for pediatric IBMFS, achieving high engraftment rates and acceptable survival outcomes. However, the high incidence of GVHD and CMV reactivation underscores the need for refined conditioning regimens and enhanced post-transplant care. The predominance of FA in this cohort highlights disease-specific challenges, suggesting that tailored protocols may improve outcomes. Future research should focus on optimizing donor selection, minimizing GVHD, and exploring novel immunosuppressive strategies to enhance long-term survival and quality of life in these vulnerable patients.

Session 2 -Pediatric SCT（non-malignancy）

Primary HLH/PID

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Primary HLH is caused by a group of genetic mutations associated with immune dysfunction which lead to persistent activation of cytotoxic T, NK cells and macrophages and cytokine storm. Allogeneic HSCT can effectively control the development of primary HLH and early genetic testing to identify gene abnormalities for diagnosis is recommended. Treatment to control excessive inflammation is of paramount importance prior to replacing the defective immune system by HSCT. Use of newer agents such as alemtuzumab, targeting cytokines and JAK/STAT blockade are promising approaches in addition to the traditional HLH 2004 protocol. Improvements in controlling the disease prior to HSCT and advances in techniques for HSCT have resulted in much better outcomes. Lack of a fully tissue-type matched donor is nolonger a barrier to HSCT in specialised centres.

SESSION 3

LONG-TERM FOLLOW-UP

Session 3-Long-term follow-up

Telehealth delivered non-pharmaceutical treatment program to improve the long-term health of patients post Haematopoietic Stem Cell Transplant

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Advances in haematopoietic Stem Cell Transplant (HCT) has increased the number of survivors needing long-term support. The lifespan of transplant recipients who survive over 2-year post-HCT approaches the age-matched population. Many are burdened with transplant-related complications and common chronic diseases including cardiovascular disease and metabolic disorders leading to increased healthcare costs and reduced survival, quality of life and economic independence. Meeting the health needs of increasing HCT survivors remains challenging. In-person programs of physical exercise and mindfulness-based stress management (MBSM) have been shown to benefit cancer patients in early treatment phase. However, the durability of benefits and delivery via the Internet especially to transplant survivors remain unaddressed. Our recent trials are the first reports that demonstrated the feasibility, safety and sustained benefits of a virtually supervised exercise and MBSB program. The use of digital health tools to support delivery and adherence further enhances scalability and real-world applicability. This program would reduce healthcare burden and support clinicians in delivering comprehensive, person-centred care to HCT survivors.

Session 3-Long-term follow-up

SURVIVORSHIP AFTER ALLOGENEIC STEM CELL TRANSPLANTATION: QUALITY OF LIFE AND FACTORS ASSOCIATED WITH RETURN TO WORK

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Background: Experience on long-term survivorship and late effects after allogeneic hematopoietic stem cell transplantation (Allo-HSCT) have greatly improved during the last decade... There is a wide burden of morbidity ranging from clinical, psychological and cognitive impairment s, with considerable impact on health and quality of life. This implies a great need for a standardized approach in the management of long-term survivors.
With longer follow-up time since Allo-HSCT, long-term survivors no longer receive direct care from their transplant center but are regularly controlled by their onco-hematologist or primary care provider.

Unmet Needs: The success of the long-term survivorship care depends not only on transplantation success but also on survival with quality of life (QoL) and capacity to return to work (RTW) when applicable. Chronic GVHD and management of its late adverse events are quite well defined.. However post-transplant mental health and cognitive functions have significant impacts on patients' ability to RTW and influence daily activities. Understanding these factors is essential to optimizing long-term outcomes after Allo-HSCT.

Observations: we analyzed retrospectively 127 transplanted patients, in remission one Year post Allo-SCT, who accepted to fill questionnaires . Of these, 78% had worked before transplantation - 43% female and 57% male. The median age was 47 (18-65) years. Women were more likely to experience anxiety ($p = 0.004$) with 45.7% of those who did not RTW, reporting this issue ($p = 0.03$). European patients had higher depression rates compared to non-Europeans ($p = 0.047$). Married/partnered patients showed higher cognitives scores (Cog)-QoL levels than those divorced/separated ($p = 0.03$), with a similar trend in the employed subgroup ($p = 0.059$). Patients who resumed cultural, travel or leisure activities after HSCT had significantly lower anxiety ($p = 0.01$, $p = 0.04$, $p = 0.003$) and depression ($p = 0.02$, $p = 0.02$, $p = 0.002$) scores.

This population had also higher cognitive (Cog)-QoL levels ($p = 0.01$, $p = 0.02$, $p = 0.04$), and RTW was correlated with improved Cog-Qol scores ($p = 0.0054$, $p = 0.0016$).

Recommendations: Beside management of cGVHD and cancer screening**,** these findings emphasize the importance of addressing psychological and cognitive health evaluation in post-transplant care to facilitate RTW and restoration of quality of life in daily activities.

Session 3-Long-term follow-up

International recommendations for screening and preventative practices for long-term survivors of transplantation and cellular therapy: a 2023 update

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With over half a million hematopoietic cell transplantation (HCT) survivors expected in the USA in 2030 (Majhail BBMT, 2013), and therefore even more worldwide, eleven major HCT scientific societies (CTTC, ASTCT, CIBMTR, COG, PTCTC, LABMT, SBTMO, EBMT, EMBMT, APBMT and ANZTCT) decided to join forces to update de original long-term survivorship guidelines initially published in 2006. The literature was extensively reviewed and the quality of available evidence was graded and categorized based on the National Comprehensive Cancer Network (NCCN) approach by the group of experts participating in the initiative. This presentation will summarize the general recommendations regarding all major organ toxicities, subsequent malignant neoplasms, psycho-social and quality of life issues that can be expected after HCT. Vaccination guidelines will be reviewed, as well as current patient peer support options. Special attention will also be given to sub-group populations such as non-malignant diseases, auto-immune disease, multiple myeloma and amyloidosis, geriatric populations and adolescents & young adults.

SESSION 4

CAR-T CELL THERAPY CLINICAL ASPECT

Session 4-CAR-T Cell Therapy Clinical Aspect

Real world experience of sourcing & establishing CART service in a resource limited centre in our region, The Hong Kong story

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CAR T-cell therapy represents a paradigm shift in cancer treatment. It has revolutionized the treatment of B-cell malignancies and multiple myeloma. Yet, we face significant accessibility challenges. According to an APBMT survey performed in 2022, CAR T-cell therapy was available in only 9 of 19 countries/regions among the 22 countries/regions participating in APBMT as of Apr 2022.
In Hong Kong, the first commercial CAR-T product was registered for clinical use in 2021. Queen Mary Hospital was the first hospital in the territory to offer this treatment to eligible patients through a pilot prioritization program. Here, we share our experience as we faced the barriers of high costs, complex workflow logistics, patient referrals and care coordination, etc in an already busy haematopoietic stem cell transplantation center.

Session 4-CAR-T Cell Therapy Clinical Aspect

International referral and cooperation for difficult HSCT and CAR-T cell therapy: Example in Taiwan

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The Asia-Pacific Blood and Marrow Transplantation Group (APBMT) currently comprises 23 countries or regions addressing a wide range of issues related to Hematopoietic Stem Cell Transplantation (HSCT) in the Asia-Pacific region. While some countries have adopted advanced technologies to perform various types of HSCT, certain countries or regions still face challenges in implementing complex procedures such as HSCT from mismatched related or unrelated donors, treating very young or elderly patients, managing advanced hematologic malignancies, or applying novel therapies such as chimeric antigen receptor (CAR) T-cell therapy.
Taiwan, one of the founding members of the APBMT since 1990, has developed a modest yet steadily growing HSCT program. In recent years, referrals of international patients from neighboring countries to Taiwan have increased substantially. For instance, Hualien Tzu Chi Hospital, located in eastern Taiwan, has provided HSCT and CAR T-cell therapy to patients from Indonesia, Malaysia, Myanmar, the Philippines, and Vietnam.
Numerous challenges have been encountered, including language barriers, cultural differences, financial constraints, psychological support, nursing care, treatment outcomes, and the need for seamless coordination with the referring institutions post-treatment. This report aims to share our recent experience and insights in delivering HSCT and CAR T-cell therapy to international patients in Taiwan.

Session 4-CAR-T Cell Therapy Clinical Aspect

Aiming for More Appropriate Management of Complications in Chimeric Antigen Receptor (CAR-)T Cell Therapy

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Over the past decade, chimeric antigen receptor (CAR-) T cell therapy has emerged as a powerful and effective treatment for patients with hematopoietic malignancies. Until now, Kyoto University has performed CAR-T cell therapy in over 250 cases, making it one of the leading facilities in Japan. Based on the large number of clinical experiences, we have conducted extended researches on CAR-T cell therapy in various aspects, including (1) patient selection, (2) lymphocyte apheresis, (3) bridging therapy, (4) management of either acute or long-term complications. In this session, I would like to give a presentation about the management of complications in CAR-T cell therapy by introducing three studies we have reported so far. First, we identified the level of phosphate as an early biomarker that can predict the development of cytokine release syndrome (CRS). Second, we have found cranio-cervical edema occur shortly after the onset of CRS and defined the phenomenon as an acute minor complication of CAR-T cell therapy. Lastly, we have established a new predictive scoring model for prolonged hematotoxicity, the "KyoTox a-score." This scoring model is based on severity of inflammation shortly after CAR-T infusion, and can predict the onset and the duration of prolonged hematotoxicity more accurately than the conventional predictive model. CAR-T cell therapy continues to evolve in both clinical and basic research fields, and will be applied to more and more patients with a wider variety of diseases in the future. We believe that our research contributes to maximizing the great potential of CAR-T by ensuring its safety.

SESSION 5

SCT COMPLICATION-INFECTION

Session 5-SCT Complication-Infection

Strategies for Preventing and Managing HBV Reactivation after Allogeneic HSCT

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Hepatitis B virus (HBV) reactivation remains a major concern in recipients of allogeneic hematopoietic stem cell transplantation (allo-HSCT) with resolved HBV infection. While HBV-DNA-guided preemptive nucleos(t)ide analog (NA) therapy effectively reduces the risk of HBV-related hepatitis, strategies for safely discontinuing NAs and preventing recurrent reactivation remain unclear. We conducted a nationwide retrospective study in Japan to assess outcomes after NA cessation in 72 patients who developed HBV reactivation post-allo-HSCT. Among them, 24 discontinued NAs during follow-up, and 46% experienced a second HBV reactivation. The risk of recurrence was higher in patients with low anti-HBs titers at NA cessation. Multiple reactivations were also observed in some cases, underscoring the need for continued HBV-DNA monitoring and the potential importance of protective anti-HBs levels for safe NA discontinuation.

To explore immunization as a preventive strategy, we conducted a prospective randomized controlled trial (PREVENT HBV) to assess the efficacy of post-transplant hepatitis B (HepB) vaccination. At day 140 after allo-HSCT, 64 patients were randomized to receive the HepB vaccine or no intervention. Although the difference in HBV reactivation rates was not statistically significant between groups (18% vs. 29%), no reactivations occurred after patients achieved a successful immune response to the vaccine. These findings suggest that effective HBV vaccination post-transplant may provide long-term protection.

In conclusion, our studies support a multipronged strategy combining vigilant HBV-DNA monitoring, preemptive NA therapy, and post-transplant vaccination to manage HBV reactivation risk. Even if HBV reactivation occurs, preemptive NA therapy can prevent hepatitis, and once vaccine-induced immunity is achieved, NAs may be safely discontinued without significant risk of further reactivation.

Session 5-SCT Complication-Infection

BK polyomavirus infection after hematopoietic cell transplantation

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BK polyomavirus (BKPyV) is a member of the genus *Polyomavirus*, a pathogen that is well recognized to cause hemorrhagic cystitis and nephritis, mainly after hematopoietic cell transplantation (HCT) or kidney transplantation. Primary infection with BKPyV is considered to occur in early childhood and thus a seropositivity rate reaches up to 90% in children 5 to 9 years of age. On the basis of such high seropositivity, BKPyV-associated diseases are considered to occur as the reactivation of intrinsic BKPyV under an intensive immunosuppressive condition such as HCT. Since the transmission routes of BKPyV remain unelucidated, however, nosocomial infection is possible among the recipients of HCT. Therefore, we have evaluated the PCR products of BKPyV DNA obtained from 9 patients developing HC due to BKPyV during a 6-month period in a Hematology/HCT ward. Six had subtype I, 2 subtype IV, and 1 subtype II or III. In the alignment of sequences, four and two of the six subtype I strains were completely homologous, suggesting a nosocomial infection. We also evaluated the BKPyV viremia after allogeneic HCT. The cumulative incidence of BKPyV viremia was 27.9%. BKPyV viremia itself did not affect post-transplant estimated glomerular filtration rate (eGFR); however, BKPyV viremia of high viral load was significantly associated with decreased eGFR. In addition, BKPyV viremia was associated with a significantly lower progression-free survival at 3 years (35.1% vs. 60.4%). The findings in our study suggest that BKPyV viremia was associated with the negative impact of renal function and survival after allogeneic HCT. We will summarize our findings of BKPyV infection after HCT and propose future studies to further elucidate its pathogenesis.

Session 5-SCT Complication-Infection

RESULTS OF APHERESIS GRANULOCYTE TRANSFUSION APPLICATION FOR TREATING NEUTROPENIC PATIENTS WITH SEVERE INFECTION AT THE NATIONAL INSTITUTE OF HEMATOLOGY AND BLOOD TRANSFUSION FROM 2019 TO 2024

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National Institute of Hematology and Blood Transfusion

Background: In the treatment of hematological diseases, the neutropenic phase may lead to severe infection and a high risk of death in patients. Granulocyte transfusion (GT) is an effective solution for patients with severe infections, especially in cases of multiple drug resistance. However, the role of this method is not universally agreed upon in many studies. Therefore, it is crucial to conduct related studies in clinical practice to gather more evidence regarding the effectiveness of this method.
Objectives: To analyze the results of applying apheresis GT for the treatment of neutropenic patients with severe infections at the National Institute of Hematology and Blood Transfusion.
Subjects and methods: A retrospective, longitudinal study of 26 patients who underwent allogeneic stem cell transplantation and were diagnosed with neutropenia and severe infection, receiving 65 apheresis GT in the Stem Cell Department, NIHBT, from 2019 to 2024. Patient characteristics, granulocyte units, and improvements in clinical and laboratory indices were analyzed.
Results: Of the patients, 80.8% were diagnosed with sepsis, among whom 85.7% experienced two or more infectious pathogens, including bacteria or invasive fungi. Among the 26 patients, 11 developed septic shock and seven faced rejection. The median duration from the conditioning regimen to neutropenia was 10.5 days, from febrile neutropenia diagnosis to GT was nine days, and neutrophil recovery lasted 14 days. The median neutrophil count in each unit was 2.56 × 10^10 cells. Each transfusion resulted in a median increase in neutrophil count of 0.52 × 10^9/L, and there was a 1-point decrease in the SOFA score. The initial response rate was 80%, with survival rates following GT of 92.4% after seven days and 88.5% after 30 days, respectively. Among the seven patients who experienced graft rejection, three were effectively treated with salvage transplantation supported by GT. Mortality is primarily attributed to a lack of engraftment. Key factors associated with successful GT treatment included

earlier initiation of GT, absence of septic shock before GT, fewer than two pathogens, decreased SOFA score, and presence of engraftment.
Conclusions: The application of apheresis GT has a high response rate and leads to an improvement in infection status in patients with engraftment potential.

SESSION 6

CAR-T CELL THERAPY LABORATORY ASPECT

Session 6-CAR-T Cell Therapy Laboratory aspect

Development of piggyBac transposon method, a non-viral gene delivery platform, for CAR-T manufacturing for clinical trials

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Background: Chimeric antigen receptor-modified T cells targeting CD19 (CD19.CAR-T cells) have shown clinical success in patients with hematological malignancies. However, a major concern for its global spread, especially in developing countries, is the high cost of CAR-T manufacturing.

Methods: We developed a method of non-viral gene transfer using *piggyBac* transposon to reduce the cost of CAR-T cell production. We started a human clinical trial to define feasibility, toxicity, maximum tolerated dose and clinical response of CD19.CAR-T cells in patients with relapsed or refractory B-ALL (jRCTa040190099). We engineered autologous T cells via the piggyBac transposon system with CD19.CAR-expression transposon vector and piggyBac transposase-expression vector to express CD19.CAR incorporating CD28 costimulatory domain. We designed this phase I trial using a modified 3 + 3 design to enroll 12 patients with relapsed or refractory acute lymphoblastic leukemia in both children and adults. In this study, patients in cohorts 1 (16-60 years old) and 2 (1-15 years old) receive 1×10^5 CAR-transduced T cells per kg. Patients in cohorts 3 and 4 (1-60 years old) receive 3×10^5 and 1×10^6 CAR-transduced T cells per kg, respectively.

Results: None of the patients had dose-limiting toxicities (DLT) in cohort 1,2 and 3. Expansion of piggyBac CAR-T cells was observed in the peripheral blood of all patients treated with the drug. Two patients in cohorts 1 and 2, which received the lowest dose, experienced recurrence, but no recurrence has occurred in cohorts 3 and 4 to date, and the 2-year disease-free survival rate and survival rate were 81.0%. Nagoya University signed a material transfer agreement to support piggyBac transposon mediated CAR-T cell therapy programs with Chulalongkorn University in Thailand and BTH hospital in Vietnam respectively.

Conclusions: PiggyBac transposon, a non-viral vector system, could be used for the production of CAR-T cells. Especially in Asian developing countries, it is important to reduce the manufacturing cost of CAR-T cells.

Session 6-CAR-T Cell Therapy Laboratory aspect

CAR T-cell Therapy in Resource Limited Setting

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While outcomes of B- Acute Lymphoblastic Leukemia (B-ALL) in children, adolescents and young adults has greatly improved over the decades to consistently over 95% in the developed countries, it hovers around 70-75% in more resource limited settings in nations like India. The critical difference now comes from the management of relapsed/refractory diseases. White allogenic stem cell transplant (Allo-SCT) has been the standard option for patients in this situation, it is highly resource- intensive requiring infrastructure highly trained personnel and extensive supportive care making it challenging to deal with the high volumes of patients that we deal with. Alternative and complimentary strategies are thus even more needed in lower resource settings. The success and high-response rates of CAR T-cell cell therapy in r/r B- ALL provided an appealing solution. The challenge was to be able to replicate the success of developed countries indigenously and at an affordable cost.

With this aim, we embarked on a project to develop a novel CD19 -directed CAR T-cell product manufactured by semi- automated processes that could be taken to early-phase clinical trials. This was a collaborative endeavor of Tata Memorial Center & the Indian Institute of Technology in Mumbai, and subsequently the technology was licensed out to ImmunoACT. Based on the success of the phase 1 & 2 trials, the product is now approved for use in relapsed/ refractory B- ALL and B- NHL. The clinical trials, since published, have reported high efficacy and low toxicity rates and the product is available at a cost of less than USD 30000, making it widely accessible and a viable option for many patients. Key results from the pre-clinical studies and clinical trials will be presented along with a suggested algorithm for use in r/r B- ALL. The development of of this first Cell and Gene therapy in India has been followed by a rapid increase in the number of clinical trials of novel and collaborative products. A healthy ecosystem now exists with infrastructure and expertise to develop new therapies through the R & D stage right though manufacturing, clinical trials and commercialization. The government is also now taking a more pro-active role in supporting innovation with a big thrust for bio manufacturing making this an exciting time for the growth of Cell & Gene therapy in the region.

Session 6-CAR-T Cell Therapy Laboratory aspect

POINT-OF-CARE MANUFACTURING OF CAR-T CELL THERAPY IN VIETNAM: OPPORTUNITY - CHALLENGE – SOLUTION

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Chimeric antigen receptor (CAR) T-cell therapy represents a significant advancement in adoptive cell transfer immunotherapy by genetically modifying T cells. This innovative therapy is poised to become a powerful instrument for treating otherwise incurable hematological malignancies, supplementing existing medical treatments. To date, CAR T-cell therapy has been approved for treatment in relapsed or refractory B-cell malignancies, including B-cell acute lymphoblastic leukemia (B-ALL), non-Hodgkin lymphoma (NHL), and multiple myeloma (MM). Currently, the most prevalent targets in CAR T-cell therapy include CD19 and BCMA, while a range of novel therapeutic targets is under investigation. As of now, the Food and Drug Administration (FDA) has authorized seven distinct CAR T-cell therapies that are commercially available. However, the high cost of these products, estimated at approximately half a million dollars per dose, presents a considerable financial obstacle for economically disadvantaged patients and individuals in developing countries. In response to this issue, in-house CAR T-cell therapy utilizing point-of-care (PoC) manufacturing emerges as a viable alternative for countries such as Vietnam. This PoC model offers several advantages compared with commercial manufacturing, including reduced costs, shortened production timelines, increased accessibility, and enhanced patient convenience. There are various methods for in-house CAR T-cell manufacturing, categorized by the type of equipment used, such as automated, semi-automated, and manual processes. On the other hand, there are two main approaches for transgene delivery in CAR-T cell manufacturing: viral and non-viral. While viral transduction methods demonstrate high transfection efficacy, they are associated with significant costs. Conversely, non-viral gene delivery methods, including CRISPR/Cas9, transposons, and mRNA transfection, are cost-effective but typically exhibit lower transfection rates. Recent studies have indicated improvements in the transduction efficiency of non-viral methods. In light of these promising advancements, the PoC manufacturing of CAR T-cell therapy using non-viral methods emerges as a viable solution, presenting opportunities for the treatment of patients with hematological malignancies in Vietnam.

SESSION 7

PEDIATRIC SCT (malignancy)

Session 7-Pediatric SCT (malignancy)

CMV infection in Pediatric Hematopoietic Stem Cell Transplantation in Low & Middle-Income Countries

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Cytomegalovirus (CMV) establishes lifelong latency after primary infection and can periodically reactivate, often with viral shedding. In immunocompetent individuals, both primary and recurrent infections are typically asymptomatic or mild. However, in immunosuppressed allogeneic hematopoietic stem cell transplant (allo-HSCT) recipients, particularly those undergoing HLA-haploidentical transplants, CMV reactivation can cause serious morbidity, mortality, and increased healthcare costs.

In low- and middle-income countries, especially in Asia, CMV poses a significant challenge in the management of children undergoing HSCT due to high seroprevalence, limited access to newer antivirals (e.g., letermovir, maribavir), and a lack of pediatric-specific data. Diagnostic limitations— such as the inability to perform genotypic testing for UL97 or UL54 mutations— can delay identification and management of ganciclovir-resistant CMV strains. The financial burden of CMV treatment is considerable, particularly in settings without comprehensive subsidies. In Malaysia, a two-week hospital stay for antiviral therapy can cost around USD 2,400, often paid out-of-pocket. Second-line drugs like foscarnet (USD 280/vial) and cidofovir (USD 1,500/vial) further increase costs, limiting patient access to optimal care.

Beyond drug costs, CMV reactivation incurs broader economic impacts, including prolonged hospitalization, additional testing, and loss of parental income. Our findings suggest that prophylactic use of anti-CMV agents in high-risk patients may be justified. Despite higher initial costs, such strategies could improve outcomes and reduce overall resource use.

Session 7-Pediatric SCT (malignancy)

Hematopoietic Stem Cell Transplantation in Pediatric Acute Leukemia at Ho Chi Minh City Blood Transfusion and Hematology Hospital, Vietnam

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Background: Acute leukemia is the most common pediatric malignancy. Although chemotherapy remains the mainstay of treatment, relapse continues to pose a major challenge. Hematopoietic stem cell transplantation (HSCT), including allogeneic (allo-HSCT) and haploidentical (haplo-HSCT) modalities, provides a curative option for high-risk or relapsed patients, especially those lacking fully HLA-matched donors.
Objective: To evaluate the initial treatment outcomes of allo-HSCT and haplo-HSCT in pediatric patients with acute leukemia at the Blood Transfusion and Hematology Hospital in Ho Chi Minh City, Vietnam.
Methods: This retrospective case series included 45 pediatric patients with acute leukemia who underwent HSCT between January 2017 and December 2024. Thirty-two patients received allo-HSCT, while thirteen received haplo-HSCT. All patients were transplanted with peripheral blood stem cells. Donor sources included matched siblings, parents, and one matched unrelated donor.
Results: Engraftment was achieved in 30 of 32 allo-HSCT patients and 11 of 13 haplo-HSCT patients. Median times to neutrophil and platelet recovery were 16 and 18 days (allo-HSCT), and 18 and 21 days (haplo-HSCT), respectively. Post-transplant infections were reported in over 90% of patients. Two transplant-related early deaths occurred in each group. After a median follow-up of 23 months (range: 2–70), the estimated 2–3-year overall survival (OS) and event-free survival (EFS) rates were 72% and 69% in the allo-HSCT group, and 60% and 55% in the haplo-HSCT group.
Conclusion: HSCT is a safe and effective treatment for pediatric acute leukemia. Haploidentical transplantation is a promising alternative for patients without fully matched donors.

Session 7-Pediatric SCT (malignancy)

A nationwide phase II study of delayed local treatment for children with high-risk neuroblastoma: The Japan Children's Cancer Group Neuroblastoma Committee Trial JN-H-11 and JN-H-15

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Neuroblastoma Committee of Japan Children's Cancer Group (JCCG)

Background:
Neuroblastoma Committee (JNBSG) of Japan Children's Cancer Group (JCCG) conducted a phase II nation-wide clinical trial, JN-H-11 and JN-H-15, for high-risk neuroblastoma. These protocols were characterized by "delayed local treatment" in which tumor resection was performed after completing all chemotherapeutic courses including myeloablative high-dose chemotherapy (HDC).
Patients and method:
Seventy-five patients with high-risk neuroblastoma were enrolled in JN-H-11 between May 2011 and September 2015 and sixty-five patients were enrolled in JN-H-15 between February 2015 and March 2018. Myeloablative chemotherapies consisted melphalan, etoposide, and carboplatin for JN-H-11 and busulfan and melphalan for JN-H-15.
Results:
For JN-H-11, the estimated 3-year PFS and OS rate were 44.4% and 80.7%, respectively. For JN-H-15, the estimated 3-year PFS and OS rates were 56.1% and 80.3%, respectively. In JN-H-15, we evaluated Curie score at postinduction. There existed no significant difference between patients with a postinduction CS of 2 or less and more than 2 (3-year EFS, 54.8±8.9% (CS<2, n=31) vs. 36.0±16.1% (CS>=2, n=10), p=0.530). For patients with *MYCN*-amplified tumor, a nearly significant outcome difference existed by postinduction CS. Conversely, for patients with *MYCN*-nonamplified tumor, there exist no difference by postinduction CS. Delayed resections of primary tumors were performed in 50/52 patients in JN-H-11 and 41/47 patients in JN-H-15, and pathological response rates (Ef2+Ef3) were 74.4% in 43 patients in JN-H-11 and 73.7% in 38 patients evaluated in JN-H-15, respectively. Pathological response did not corelate to either the 3-year-PFS or 3-year-OS.
Conclusion:
Despite of shortness of observation time and lower numbers of enrolled patients in JN-H-15, a postinduction CS of more than 2 in patients with *MYCN*-

nonamplified tumor was not associated with poor progression-free survival. These studies indicated that delayed local treatment is feasible and showed promising efficacy, suggesting that this treatment should be considered further in a comparative study of high-risk neuroblastoma.

SESSION 8

CONDITIONING REGIMEN

Session 8-Conditioning Regimen

Treosulfan-based conditioning for allogeneic HSCT in children with non-malignant diseases

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Allogeneic hematopoietic stem cell transplantation (HSCT) has been widely used as a curative therapy for pediatric patients with non-malignant disease. Conditioning regimens play an important role in the success of HCT for pediatric patients with non-malignant diseases and have improved dramatically in recent decades. The initial HSCT approach using myeloablative conditioning (MAC) significantly improved the outcomes of patients with non-malignant disease but was associated with considerable transplant-related mortality (TRM). A subsequent strategy using reduced-intensity conditioning (RIC) remarkably reduced the incidence of TRM. However, the high level of mixed chimerism associated with RIC has prompted the search for improved conditioning regimens. Treosulfan, which is a busulfan analogue, is a prodrug and a water-soluble bifunctional alkylating agent which has been used as treatment for various cancers. Recently, treosulfan replaced busulfan as a component of a reduced toxicity conditioning regimen for HSCT. Preclinical studies demonstrated that treosulfan treatment produced a rapid and sustained myelosuppression and that this agent exhibits immunosuppressive characteristics, which contribute to stable engraftment after HSCT. In contrast to busulfan, treosulfan is also associated with fewer extramedullary toxicities, including in the liver. Recently, treosulfan has replaced busulfan as a component of a reduced toxicity conditioning (RTC) regimen. Both its myeloablative and immunosuppressive properties, as well as a favorable toxicity profile, make treosulfan a potential candidate for use as part of conditioning regimen prior to HSCT. Indeed, treosulfan-based conditioning regimens are being increasingly used in pediatric patients with various non-malignant diseases.
In this presentation, I will review the recent progress in HSCT using treosulfan-based conditioning regimen in pediatric patients with non-malignant diseases. I will also introduce the clinical experience with this approach at our center.

Session 8-Conditioning Regimen

Safety and Efficacy of VA Combined with Modified BuCy Conditioning Regimen Followed by Allo-HSCT for High-Risk or Refractory/Relapsed Acute Lymphoblastic Leukemia: A Prospective, Single-Center, Single-Arm Clinical Trial

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Background: Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is the only curative treatment option for high risk or refractory/relapsed (r/r) acute lymphoblastic leukemia (ALL). However, relapse remains a leading cause of death post allo-HSCT, with a cumulative incidence of relapse (CIR) of approximately 30%-50%. Therefore, prevention of relapse is of great importance to improve the outcome of allo-HSCT in high-risk ALL patients. Intensive myeloablative conditioning is a primary therapeutic option that can maximize the reduction of the residual leukemia burden in order to reduce disease recurrence post transplantation. Venetoclax combined with azacytidine (the VA regimen) has demonstrated synergistic anti-tumor activity against several hematological malignancies, particularly newly diagnosed and high-risk ALL (data unpublished). Furthermore, the VA regimen can disrupt energy metabolism and eliminate leukemia stem cells. Therefore, we aim to evaluate the safety and efficacy of the VA regimen combined with modified BuCy for high-risk or r/r ALL patients undergoing allo-HSCT.

Method: From Dec 2021 to Apr 2024, 20 patients diagnosed with high-risk or r/r ALL were enrolled. All patients received 7-day courses of venetoclax (200mg/d on days -17 to -11) and 7-day courses of azacytidine (75mg/m^2/d on days -17 to -11) combined with mBuCy conditioning regimen consisting of Me-CCNU 250 mg/m^2/d on day -10, cytarabine 2g/m^2 every 12h on days -9 to -8, busulfan 0.8 mg/kg every 6 h on days -7 to -5, and cyclophosphamide 1.8 g/m^2/d on days -4 to -3. For matched sibling and unrelated donors, cytarabine was given at a dose of 2g/m^2/d on day -9. Rabbit antithymocyte globulin was given at 2.5mg/kg/d on days -5 to -2 except for matched sibling donors.

Results: As of April 2024, 20 patients, with a median age of 30.5 years (ranging from 12 to 56 years),were enrolled in this study. High risk cytogenetic or molecular factors were detected in 100% patients, such as KMT2A

rearrangement, Ph-like ALL, hypodiploidy and testicular leukemia. ALL patients achieved morphological complete remission prior to transplantation, with 19 (95%) patients maintaining minimal residual disease (MRD)-negative remission. The majority (85%) received transplantation from haploidentical donors. Hematopoietic recovery was achieved for all patients, with a median time to absolute neutrophil counts (ANC) engraftment of 12 days (range: 9-13 days) and platelet (PLT) engraftment of 17 days (range: 8-30 days). During a median follow-up period of 14.5 months (range: 2.6-31.1 months), 1 patient experienced relapse at 9 months after transplantation, while another patient died from severe sepsis on day 222 post-transplantation. Overall survival (OS) at 1 year was 94.1% (95% CI, 83.6-100%). Leukemia-free survival (LFS) at 1 year was 87.8% (95% CI, 73.4-100%). The CIR at 1 year was 6.7% (95% CI, 6.7-61.6%). The non-relapse mortality (NRM) at 1 year was 5.9% (95% CI, 0-17.4%). Grade I-II acute graft-versus-host disease (aGVHD) occurred in 30% of patients, with no case of grade III-IV aGVHD. Mild chronic graft-versus-host disease (cGVHD) was observed in 3 patients. The most common non-hematologic adverse events (AEs) were grade I-II gastrointestinal issues, with diarrhea affecting 45% and nausea affecting 30% of patients. No higher-grade AEs or hepatic veno-occlusive disease (VOD) were noted. By the end of the follow-up period, the incidences of cytomegalovirus (CMV) and Epstein-Barr virus (EBV) activation were 25% (5 out of 20) and 40% (8 out of 20), respectively.

Conclusion: Our study suggests that the VA regimen combined with modied BuCy shows good tolerability, significantly decreases the relapse rate, and prolongs long-term survival in high-risk or refractory/relapsed ALL patients.

SESSION 9

HAPLO-SCT

Session 9-Haplo-SCT

Double Down: The U.S. Haplo-Cord Experience in Allogeneic

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Haplo-cord transplantation, the combination of a haploidentical donor graft with an umbilical cord blood graft, was developed to overcome the limitations of each individual platform. In the United States, this approach has been adopted to provide rapid hematopoietic recovery from the haploidentical graft, while preserving the long-term engraftment and favorable graft-versus-leukemia properties of cord blood. Over the past decade, haplo-cord has been applied in diverse patient populations, including those with high-risk hematologic malignancies and individuals from minority backgrounds with limited donor availability. Clinical experience has highlighted unique engraftment dynamics, characterized by early haploidentical “bridging” followed by durable cord blood dominance. This strategy has mitigated the problem of delayed recovery traditionally associated with cord blood transplantation, while maintaining low rates of graft-versus-host disease and extending access to transplantation. The presentation will review the U.S. experience with haplo-cord transplantation, emphasizing the biological principles, patterns of engraftment, and the clinical lessons learned. This body of work will explore the value of haplo-cord as a viable alternative donor platform and a meaningful contribution to expanding the field of allogeneic transplantation.

Session 9-Haplo-SCT

Haploidentical Transplant for Hematological Malignant Diseases: Experience at BTH Hospital

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Background: Hematopoietic stem cell transplantation (HSCT) is a curative option for various hematological malignancies and disorders. Haploidentical HSCT with post-transplant cyclophosphamide (PT-Cy) has emerged as a viable alternative for patients lacking a matched sibling or unrelated donor. This study aimed to retrospectively evaluate the outcomes of haploidentical HSCT with PT-Cy at BTH Hospital.
Methods: We retrospectively analyzed data from the patients who underwent haploidentical HSCT with PT-Cy at BTH Hospital between February 2016 and April 2025. Outcomes, including overall survival (OS), progression-free survival (PFS), graft-versus-host-disease-free, relapse-free survival (GRFS), and cumulative incidences of acute and chronic graft-versus-host disease (GVHD), relapse, and non-relapse mortality (NRM) were analyzed.
Results: There were 69 patients who underwent haploidentical HSCT with PT-Cy at BTH Hospital between February 2016 and April 2025. The study population included patients with AML (n=47), MDS (n=4), ALL (n=11), MPNs (n=4), MDS/MPNs (n=2), and HLH (n=1). Conditioning regimens were myeloablative (n=35) or reduced-intensity (n=34). The median follow-up duration was 40 months. At 3 years, the estimated rates of OS, PFS, and GRFS were 51.4 ± 6.7%, 41.1 ± 6.5%, and 41.3 ± 6.5%, respectively. The cumulative incidence of acute GVHD at 100 days was 27.5%, while the cumulative incidence of chronic GVHD at 1 year was 23.9%. Cumulative incidences of relapse and NRM at 1 year were 21.3% and 20.3%, and at 3 years were 32.3% and 21.8%, respectively. Univariate analysis identified two factors adversely affecting OS: female donor to male recipient (HR 2.74, p=0.02) and donor age ≥ 37 years (HR 2.13, p=0.037). Female donor to male recipient was also a significant adverse factor for PFS in univariate analysis (HR 2.32, p=0.046). In multivariate analysis, donor age ≥ 37 years was an independent adverse risk factor for both OS (HR 3.27, p=0.004) and PFS (HR 2.44, p=0.018). The leading causes of death were relapse (54%) and post-transplant infection (30%). These outcomes are comparable to findings from other international studies.
Conclusion: Haploidentical HSCT with PT-Cy is a feasible and effective treatment for a diverse group of patients with hematological diseases. Donor age and donor-

recipient sex mismatch are important factors influencing outcomes. Future studies are needed to further optimize donor selection and patient management.

Session 9-Haplo-SCT

Less is More: The Evolution of PTCy Dose for Safer Allogeneic Transplantation

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Post-transplant cyclophosphamide (PTCy) has revolutionized graft-versus-host disease (GVHD) prophylaxis, transforming the landscape of allogeneic hematopoietic stem cell transplantation (HCT). Initially developed for haploidentical HCT, its use has rapidly expanded to become a standard of care in transplants from matched related (MRD), matched unrelated (MUD), and mismatched unrelated donors (MMUD). Despite its broad success, the standard 100 mg/kg dose is associated with significant toxicities, particularly myocardial damage. This universal concern has made dose optimization a critical goal to improve the safety of this platform.

In Japan, we have systematically addressed this issue. Our initial prospective phase II studies in the haploidentical setting (Sugita et al., *Bone Marrow Transplant* 2021) established the safety and feasibility of a reduced 80 mg/kg PTCy dose. Subsequently, a large-scale Japanese registry analysis (Fuji et al., *British Journal of Haematology* 2024) provided definitive evidence. This analysis showed that the 80 mg/kg dose offered comparable efficacy to the 100 mg/kg standard. Notably, deaths from cardiac complications within 30 days of transplant occurred in 7 of the 425 patients in the standard-dose group versus none in the 425 patients in the reduced-dose group, highlighting a significantly improved safety profile.

These findings, primarily derived from haplo-HCT, provide a powerful rationale for dose de-escalation across all PTCy-based platforms, spurring a global initiative to validate this less-toxic approach in prospective trials. This presentation will review the compelling evidence from Japan that supports this paradigm shift, and discuss the global effort to establish a new, less toxic standard of care for GVHD prophylaxis in allogeneic HCT.

SESSION 10

SCT COMPLICATION-GVHD

Session 10-SCT Complication-GVHD

Low dose PT-Cy as GVHD prophylaxis

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Many studies have been conducted on the effectiveness of high-dose post-transplantation cyclophosphamide (PTCy) in preventing graft-versus-host disease (GVHD). However, few studies have focused on low-dose post transplant cyclophosphamide in related, unrelated, or haploidentical settings. In this presentation, we will report our data, starting with immunology and clinical implementation in limited resource settings, as well as preliminary results from a prospective randomized multicenter study. We will discuss immunoblation efficacy for various doses of PTcy compared to other GVHD prevention regimens. Next, we present preliminary clinical data showing that different doses yield similar results, followed by discussion on how to implement this approach in resource-limited settings. Finally, the results of a prospective randomized multi-center study will confirm no difference in aGVHD rates.

Session 10-SCT Complication-GVHD

Recent Advances in GvHD Research: From Bench to Bed

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Allogeneic haematopoietic stem-cell transplantation (allo-HSCT) can cure many haematological malignancies, yet acute graft-versus-host disease (aGvHD) remains the leading cause of non-relapse mortality. Conditioning-related intestinal injury and microbiota disruption further complicate its management and limit the predictive value of pre-clinical models.

To uncover druggable pathways for aGvHD prevention, we integrated transcriptomic profiling with functional screening of murine and patient-derived allogeneic T cells. We identified the de-ubiquitinase OTUD1, which stabilises the Notch2 intracellular domain (NICD) by de-ubiquitylating K1770, thereby amplifying $CD4^+$ T-cell activation and accelerating aGvHD. In silico drug discovery revealed that the SGLT2 inhibitor dapagliflozin selectively targets the OTUD1–NICD axis, restraining T-cell activation and effector function and prolonging survival. These findings have led to a phase II prophylactic trial of dapagliflozin (NCT06626737) and a phase III trial of the DPP-4 inhibitor sitagliptin (NCT05149365) in the allo-HSCT setting.

Concurrently, multi-omics analyses showed that type VI secretion system (T6SS)–mediated interbacterial antagonism reshapes gut microbiota composition and the intestinal metabolome—most notably the bile-acid pathway. This ecological re-engineering attenuates mucosal inflammation, preserves epithelial-barrier integrity, and mitigates aGvHD. These insights prompted a randomised trial of autologous faecal microbiota transplantation (FMT) for severe steroid-refractory intestinal aGvHD (NCT04745221), which achieved high response rates while maintaining graft-versus-leukemia activity.

Together, the OTUD1/Notch and T6SS–bile-acid axes represent complementary checkpoints in allo-immunity and host–microbe crosstalk. Their simultaneous modulation provides a rational, mechanism-driven strategy to prevent or treat aGvHD. This work exemplifies an integrated bench-to-bedside pipeline that translates molecular discoveries into first-in-human interventions, accelerating progress toward safer and more effective allo-HSCT.

Session 10-SCT Complication-GVHD

GVHD Update EBMT Guidelines

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Graft-versus-host disease (GVHD) is a major factor contributing to mortality and morbidity after allogeneic haematopoietic stem-cell transplantation (HSCT). In the last 3 years, there has been regulatory approval of new drugs and considerable change in clinical approaches to prophylaxis and management of GVHD. To standardise treatment approaches, the European Society for Blood and Marrow Transplantation (EBMT) has updated its clinical practice recommendations. We formed a panel of one methodologist and 22 experts in the field of GVHD management. The
selection was made on the basis of their role in GVHD management in Europe and their contributions to the field, such as publications, presentations at conferences, and other research. We applied the GRADE process to ten PICO (patient, intervention, comparator, and outcome) questions: evidence was searched for by the panel and graded for each crucial outcome. In two consensus meetings, we discussed the evidence and voted on the wording and strengths of recommendations. Key updates to the recommendations include: (1) primary use of ruxolitinib in steroid-refractory acute GVHD and steroid-refractory chronic GVHD as the new standard of care, (2) use of rabbit anti-T-cell (thymocyte) globulin or post-transplantation cyclophosphamide as standard GVHD prophylaxis in peripheral blood stem-cell transplantations from unrelated donors, and (3) the addition of belumosudil to the available treatment options for steroid-refractory chronic GVHD. The EBMT proposes to use these recommendations as the basis for routine management of GVHD during allogenic HSCT.

SESSION 11

LYMPHOMA / MYELOMA

Session 11-Lymphoma/Myeloma

Outcomes of Autologous Stem Cell Transplantation in Japanese Patients Aged ≥65 Years with Relapsed or Refractory DLBCL: A Nationwide Analysis in the Era of CAR-T Therapy

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Background: The rising incidence of relapsed or refractory diffuse large B-cell lymphoma (R/R DLBCL) among older populations poses a critical challenge in Japan's super-aging society. While high-dose chemotherapy followed by autologous stem cell transplantation (ASCT) has long been the standard of care for chemosensitive R/R DLBCL, real-world evidence in patients aged ≥65 years remains limited, especially with the recent emergence of CAR-T cell therapy as an alternative option.
Objective: To evaluate clinical outcomes and identify prognostic factors for ASCT in Japanese patients aged ≥65 years with R/R DLBCL, and to clarify the role of ASCT alongside novel cellular therapies.
Methods: We retrospectively analyzed data from the nationwide TRUMP registry, enrolling 451 patients aged ≥65 years who underwent first ASCT for R/R DLBCL (CR2 or PR1) between 2011 and 2022. Outcomes were assessed up to the introduction of alternative therapies (allo-SCT/CAR-T). Multivariate analyses evaluated predictors for non-relapse mortality (NRM), relapse, progression-free survival (PFS), and overall survival (OS).
Results: The median age was 67 years (range, 65–80). Three-year OS was significantly higher in patients with >24 months from diagnosis to ASCT (73.4%) compared to ≤24 months (58.6%, $p<0.01$), and in those with ECOG performance status (PS) 0 at ASCT (69.4% vs. 60.6%, $p=0.02$). HCT-CI >0 and male sex were independent predictors for NRM. The uptake of ASCT plateaued after 2018, coinciding with the approval of CAR-T cell therapy in Japan. Most relapses occurred within 24 months post-ASCT.
Conclusion: ASCT remains an effective and safe option for highly selected older patients with chemo-sensitive, late-relapsed DLBCL, particularly those with good PS and minimal comorbidities. Individualized, evidence-based treatment selection is essential, considering emerging alternatives such as CAR-T therapy. These findings inform optimal management strategies for older adults with R/R DLBCL in Asia's aging society.

Session 11-Lymphoma/Myeloma

Advances in the Management of Multiple Myeloma

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The therapeutic landscape of multiple myeloma continues to evolve rapidly, driven by innovations in frontline regimens and immune-based therapies. This presentation offers a contemporary overview of key advances as of 2025, with a focus on clinical relevance across diverse healthcare settings. In transplant-eligible patients, the incorporation of daratumumab into triplet induction (D-VRd) has established a new standard of care. The role of autologous stem cell transplantation remains well supported by both clinical trial data and real-world outcomes. In transplant-ineligible populations, quadruplet-based strategies are being explored to improve depth of response while balancing tolerability. The expanding use of CAR-T cell therapy and bispecific antibodies has demonstrated substantial efficacy in relapsed and refractory disease, although access disparities persist across the Asia-Pacific region. This session will also address evolving strategies for therapy sequencing, toxicity mitigation, and integration of novel agents including CELMoDs, dual bispecifics, and trispecifics. The presentation will highlight emerging therapeutic options and their implications for future treatment paradigms.

Session 11-Lymphoma/Myeloma

Revisiting the Standard 200 mg/m^2 Dose of Melphalan for Autologous Transplant in Myeloma in the Era of MRD

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Melphalan 200 mg/m^2 (Mel-200) has been the standard consolidation therapy in newly diagnosed multiple myeloma (NDMM) since the VAD (Vincristine-Adriamycin-Dexamethasone) era. Induction regimens have evolved from conventional chemotherapy based to Proteasome inhibitor (PI) plus immunomodulator drug (IMiD) based triplets to anti-CD38 based quadruplets. Similarly, maintenance has evolved from no maintenance to IMiD alone to combination of IMiD with PI or anti-CD38. While induction and maintenance strategies have seen profound transformations, Mel-200 still remains the undisputed standard conditioning dose.

Melphalan 140 mg/m^2 (Mel-140) is used in patients perceived to be at risk of excess toxicity. While transplant-related mortality (TRM) with Mel-200 ASCT remains less than 1% in western countries, it varies between 2-7% for low-middle income countries (LMICs). Moreover, the incidence of grade 3-4 mucositis in LMICs is approximately 50% with Mel-200, which contributes to patient morbidity and their perception about transplant. This highlights the need for safer and less toxic conditioning in MM. Previous retrospective analyses which have compared Melphalan 200 mg/m^2 versus 140 mg/m^2, including studies from EBMT registry, M.D. Anderson Cancer Centre (MDACC) and India, have shown similar survival for lower dose of melphalan, especially in patients who achieved at-least a VGPR prior to ASCT. Literature suggests that lower doses of Melphalan is equally effective in patients who achieve deep responses after induction. In the current era of triplets/quadruplets, majority patients achieve deep responses (≥VGPR). Our institutional analysis (n=176) of MM ASCT patients showed that there was no difference in PFS or OS between Mel-140 vs Mel-200. Importantly, when stratified for MRD (both marrow and imaging) and cytogenetics, Mel-140 was non-inferior to Mel-200 in patients with negative pre-transplant marrow / imaging MRD and those with standard risk cytogenetics. The above evidence compels us to think if time has come to dethrone Mel-200 as the “standard” conditioning in the “MRD era”!

SESSION 12

ENHANCING ACCESS TO SCT: ESTABLISHING SERVICES WITH LIMITED RESOURCES

Session 12-Enhancing Access to SCT: Establishing services with limited resources

Establishing haematopoietic stem cell transplant services with limited resources – Aurangabad, India

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With the aim to provide wider access to haematology & haematopoietic stem cell transplant (HSCT) services this report describes establishment of such services in a smaller city in India. The Kamalnayan Bajaj Charitable Trust Hospital is based in Aurangabad, Maharashtra, India & caters to approximately 5-6 million population from the city as well as neighbouring region. It's a 300 bed NABH accredited multi-speciality hospital with in-house pathology, microbiology & radiology services, paediatric & adult ICUs & radiation oncology unit equipped with TBI facility. The haematology service was started in 2015 by a single trained haematologist & extended to a HSCT program in 2017 reporting data to the Indian HSCT registry. The challenges faced were availability of irradiated blood products & establishing apheresis services - both outsourced to an accredited blood bank in the city. Collaboration with established transplant centres helped with nurse training & practices. The CD34 counts, chimerism analysis & virology monitoring are all outsourced from high throughput labs. Cryopreservation at -80C without liquid nitrogen (dump freezing) is done in house. Use of generic molecules (treosulfan,melphalan,IV busulfan,Fludarabine,ATG,Thiotepa) helped bridge drug procurement gap, although procuring defibrotide & eculizumab remain a challenge. Fundraising for poor patients keeps the program sustainable.

Results: Since inception in 2016, a total of 106 HSCTs have been performed:- Autologous: 43(myeloma 29, amyloidosis 4, lcdd 1, 6 lymphoma, 2 neuroblastoma) Allogeneic: 37(12 AML,8 SAA, 6 CML, 3ALL, 2 MDS,4 PNH,2 lymphomas,1FHLH) Haplo-identical: 26 (7AML,8 ALL,3 MDS,2 CML,3 SAA,1PNH,1lymphoma,1FHLH). Of these 90.8% patients are adults (aged 19 to 74 years) & 10/106 (9.2%) are children (age 3 to 18 years). All transplants used peripheral blood stem cell grafts for both malignant & non-malignant diseases (32 MRD, 5 MUD & 26 haplo-identical grafts). All had successful engraftment except 2 patients; 1 engrafted successfully after second transplant, while other did not survive the second transplant. Commonest indication is multiple myeloma (78%) for autologous transplant & leukaemia (48.8%) for allogeneic transplant (MRD: 43%, Haplo: 62.9%). Transplant related mortality is 4.8% for autologous transplant, 18.4% for allogeneic transplant &

35.2% for haploidentical transplant. Approximate cost of first 100 days of transplant care is 5.5 lakh INR (6500 USD) for autologous transplant, 7.2 lakh INR (8200 USD) for matched allogeneic transplant & 9.3 lakh INR (10,400 USD) for haplo-identical transplant at our centre.

Conclusion: Single physician HSCT centres can be established & sustained with appropriate institutional & peer support in overcoming challenges. This helps provide access to patients who would otherwise not reach any HSCT service. An expanded team (infectious disease specialist, onco-pharmacist, psycho-oncologists & counsellors) remains a longer term goal.

Session 12-Enhancing Access to SCT: Establishing services with limited resources

Establishing haematopoietic stem cell transplant services with limited resources – Kolhapur, India

Abhijeet Ganapule
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Introduction:
In India, Hematopoietic stem cell transplant (HSCT) services are available in tertiary care multispecialty hospitals in metropolitan cities. Access to these centers for patients from tier 2/3 cities is difficult due to financial and logistic reasons (travel and stay). If this facility is available in patient's home town, it will help in cutting the cost and add to the convenience.

Methodology:
12Drugs for conditioning chemotherapy, immunosuppression and antibiotics are widely and easily accessible in the country. In-house radiation facility is available, which has helped us to give TBI-based conditioning therapy, if needed, since the beginning of our transplant program. Inhouse hematopathology, histopathology and radiology services (including CT scan) are also available in the hospital. Microbiology services are outsourced, so are the blood bank and cryopreservation services. As this is an oncology focused hospital, services from gastroenterologist, nephrologist, dermatologist and psychiatrist are sought on consultation basis from outside. Hickman lines are expensive and the expertise being unavailable inhouse, all patients are managed with PICC lines, triple-lumen subclavian line for venous access. Nursing care is actually pillar of our transplant program. We always maintain patient to nurse ratio of 1:1. There are no medical officers or junior residents posted in transplant facility. After daily ward rounds, execution of orders, following up the reports, informing the changes in clinical condition and preparing preliminary discharge summary is done by staff nurses.

Results:
Total 29 transplants have been done (16 autologous and 13 allogeneic stem cell transplants). Of the 13 patients who underwent allogeneic stem cell transplant, 4 underwent haploidentical stem cell transplant and 9 underwent matched sibling allogeneic stem cell transplant. The commonest indication for allogeneic stem cell transplant was acute lymphoblastic leukemia (Ph positive and relapsed acute lymphoblastic leukemia) 6 cases (46%). The commonest indication for autologous transplant was multiple myeloma, 8 cases (50%). Of the 13 allogeneic stem cell transplants 4 patients are surviving, longest surviving patient post-

transplant is a case of acute myeloid leukemia for 6 years disease free and without GVHD. Causes of death in allogeneic group were as follows relapse 3(33.33%), GVHD 3(33.33%), Graft failure 1(11.11%), Sepsis with 2(22.22%). Among 16 autologous transplant patients, 9 patients are surviving, longest survivor being a case of multiple myeloma in remission after 7.5 years. Causes of death in autologous group were as follows: sepsis 4(57%) and relapse 3(43%). Transplant cost at our center is significantly low compared to larger HSCT centers in the country. Autologous transplant costs Rs 300-600,000 (US$3.5-7.2K), allogeneic stem cell transplant Rs 800-1000,000 (US$9.5-12K) whereas haploidentical transplant costs Rs. 800-1,200,000 (US$ 9.5-14 K).

Conclusion:

Now that HSCT service is established, we need to help patients to mobilize fund through CSR (Corporate Social Responsibility) and government schemes. Poor outcomes in allogeneic group were probably due to high-risk disease profile (Philadelphia chromosome positive, relapse acute lymphoblastic leukemia) and type of graft (4 of 13 allogeneic stem cell transplants were haploidentical transplants). We need to be more selective in choosing cases. In autologous group the overall survival was 56%, we can improve the outcomes in this group by reducing the transplant related mortality. One more hematologist will help in increasing as well as improving the quality of the program.

Session 12-Enhancing Access to SCT: Establishing services with limited resources

Establishing services with limited resources

Subbaiah Ramanathan
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Most of the haemopoietic stem cell transplant (HSCT) centres are established in tier-1 cities (population of 1 million and above). Establishing a HSCT centre in a tier-2 city (0.5 to less than 1 million population) remains a challenge for a new transplant physician especially in a private sector health centre. Here we share our experience in establishing a new and the first HSCT in Tiruchirapalli (a tier-2 city in Tamil Nadu, South India). I was trained in clinical haematology, bone marrow transplant services at prestigious Christian medical college, Vellore, India. Patients travelling a long distance for the same during my residency days ignited a thought to set up a haematology unit in a tier-2 city. I started here in Sep 2019. I joined a 200 bedded multi-speciality hospital and started haematology services catering adult & paediatric patients, both malignant & benign. Training nurses in oncology care, improving transfusion medicine team, expanding the scope of laboratory services were also done. We did our first autologous BMT in Aug 2020, first allogeneic BMT in March 2021. First paediatric BMT was done in Nov 2021 after a paediatric haematologist joined the team. Later we could do haploidentical and MUD BMTs. Tests like HLA typing, DSA, CD34 enumeration, chimerism analysis still are outsourced and pose a challenge for in house. We started doing BMTs in a clean single room (2020) and then established a 2 bedded HEPA filtered unit (2021) which is now upgraded to a 5 bedded unit (Sep 2024). We could do autologous BMT at 5K USD and allogeneic BMT at 10K USD. People in tier 2 cities and surrounding rural areas certainly benefit as they cannot travel often to tier 1 cities, neither afford lodging and boarding there. Till date we have done 94 BMTs with a survival of 70%.

Session 12-Enhancing Access to SCT: Establishing services with limited resources

Experience in establishing services with limited resources

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The challenge in HSCT lies in making it affordable and accessible to all patients. Training, Motivation and Support are the three essential requirements to set up a good BMT unit. Fortunately, I was trained at CMC Vellore and had total institutional support from the Muni Seva Ashram Trust. At joining, I was the only transplant physician. The unit was staffed with a few qualified nurses assisted by some who had undergone a 6-month training in a vocational college. The isolation rooms consisted of two rooms with a common bathroom. There was only a single ICU catering to medical and surgical patients. These had to be transformed into a BMT unit. Motivation and training of the available staff complemented by consistent inter-departmental cooperation have been the key to optimal utilisation of resources. Almost 9 months after joining, in a shared room without HEPA filtration or positive pressure ventilation, we did an autologous stem cell transplant. Subsequently, six transplants were carried out in the same limited facility, autologous as well as allogeneic with no peri-transplant mortality. Today we have an 8-room with HEPA filtration and positive pressure ventilation. We did a haploidentical stem cell transplant after 20 months and a matched unrelated allogeneic stem cell transplant after 4 years. For cryopreservation, a conventional blood bank deep freezer was utilised. Staffing has improved however, there is no full-time paediatrician or Infectious disease specialist. We learned through experience- sometimes painfully. For swift communication and efficient decision making, my team extensively uses WhatsApp to share patient video, radiological imaging, medication orders, vital signs and lab results in real time. We have since performed 180 Stem cell transplants with results comparable to the best centers.

SESSION 13

LEUKEMIA / MDS/ MPN

Session 13-Leukemia/ MDS/ MPN

Allogeneic HSCT in relapsed AML

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While frontline intensive- and non-intensive therapies for patients with Acute Myeloid Leukemia (AML) have improved over the past years, still relapse poses a major setback for the majority of patients. Intensive induction therapies have been shaped for fit patients with the modification of induction regimens and by adding targeted-therapies. Targeted non-intensive combinatorial treatment strategies have also been improved for elderly/unfit patients and thus converting unfit- into fit patients susceptible for potential curative allogeneic hematopoietic stem-cell transplantation (HSCT). These modifications in frontline therapy for patients with AML in combination with improvements in preparative and prophylactic measures before and during allogeneic HSCT itself result in improved fitness of patients during and after treatment, increased rates of complete remission (CR) and thus to increased rates of allogeneic HSCT worldwide. However, the incidence of relapse for all AML patient groups, including the reflection of elderly- vs. younger patients as well as fit- v. unfit patients, and various genetic risk-groups is still unacceptably high. Diagnostic-, prophylactic-, preemptive-, and therapeutic measures such as measurement of minimal residual (or measurable) disease (MRD), prophylactic- and/or preemptive treatments as well as therapeutic interventions and treatments have improved but at the same time many questions remain unanswered. The sensitivity and specificity of diagnostic measures as well as their prognostic vs. predictive impact and timing are uncertain as well as their respective degree with which they can be generalized are yet to be determined. In this presentation I will attempt to review and discuss our current understanding of the role, the path, possible modifications, and the barriers for allogeneic HSCT for patients with relapsed AML.

Session 13-Leukemia/ MDS/ MPN

FORUM trial: HSCT in children and adolescent with ALL

Yves Bertrand
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Allogenic hematopoietic stem- cell- transplantation is recommended for high-risk acute lymphoblastic (ALL) patients (1st line chemoresistant as indicated by high MRD levels after consolidation, or subsequent lines of treatment). Total body irradiation containing regimens are widely used in such patients, but TBI has lifelong many adverse effects. FORUM is a randomized, controlled, open-label, international multicenter trial investigating whether preparative combination of optimal chemotherapy is non inferior to TBI. Actualisation of the results will be presented, showing in 413 randomized patients the 4 -years EFS and OS being significantly higher following TBI versus chemoconditioning regimen (lower relapse risk and TRM).
Furthermore, other studies have been performed : chemo-conditioning with Busulfan vs Treosulfan showed no significant differences regarding EFS, OS, GvHD ; FORUM study was analysed in patients < 4y.old and in 191 evaluable children it was shown that HSCT in CR1 was better that after relapse. Multivariate analysis showed poorer EFS in children < 1 yr whereas KMT2A was significant for OS (HR 1.96).
Children with HR first relapse B-ALL receiving blinatumomab before HSCT had better EFS and OS as compared with patients treated with chemotherapy only (higher proportion of patients with undetectable MRD with immunotherapy)
Many questions are still not answered in this trial concerning the long term follow-up of patients, and particularly the late sequalae (gonadal toxicity, second malignancy, final height and organ dysfunctions).
Due to the increasing overall survival in many childhood cancers, and more than 90 % 5-years survival in ALL, it seems mandatory to integrate long-term toxicities in treatment evaluation. Recently an international initiative resulted in a consensus definition of 21 severe toxicities, and severe toxicity free-survival (STFS). This will help to compare the different toxicities across different protocols, and facilitate the modificiations of therapy with the goal of reducing toxicities without compromising the cure of the patients.

MULTIDISCIPLINARY SESSION 1

LABORATORY

Multidisciplinary Session 1-Laboratory

Model-informed precision dosing of intravenous busulfan in Thai pediatrics patients

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Introduction:
Intravenous busulfan is a cornerstone in conditioning regimens for pediatric hematopoietic stem cell transplantation. However, considerable interindividual variability in pharmacokinetics (PK) has been found. To achieve optimal busulfan exposure, (1) a covariate-based formula to predict busulfan clearance (CL) for a priori dose individualization and (2) an optimal model-based TDM strategy for a posteriori dose adjustments were determined.

Materials and Methods:
The study was conducted retrospectively. One hundred fourteen Thai pediatric patients have been recruited. Busulfan concentration data collected during TDM of patients treated in Ramathibodi Hospital (Bangkok, Thailand) were modeled with a population approach (NONMEM 7.4, Icon PLC). The influence of the following variables was screened with a stepwise covariate modeling procedure: actual age, age transformed with a maturation function, sex, malignant disease (MALIGN), fludarabine co-administration, and genetic polymorphism of Glutathione S-transferase Alpha-1 (GSTA1, rs3957357 & rs3957356). A limited sampling strategy was explored. Finally, the days when TDM should be performed were assessed through simulations with the R packages mrgsolve and mapbayr.

Results:
A mono-compartmental model with proportional residual variability is best described with IIV and IOV on CL (26.0% and 14.1%, respectively). The covariate screening revealed that CL at day 1 was best predicted a priori with the following formula: CL = (BW/25)^0.786 * 0.896^MALIGN * 0.894^GSTA1. Three concentrations (0.25, 2, and 5 hours after the end of the infusion) were sufficient for a satisfactory Bayesian estimation of CL (relative root-mean-square error: 3.4%).

Discussions and Conclusions:
Population pharmacokinetic analysis of intravenous busulfan in Thai pediatric patients suggests that body weight, the decrease of CL on days 2-3-4, diagnosis, and GSTA1 are the common predictors of CL. Moreover, this comprehensive approach

quantified the benefit of TDM to control busulfan exposure in Thai pediatric patients and suggests decreasing the number of samples to 3 per day of TDM.

Multidisciplinary Session 1-Laboratory

ASSESSMENT OF MINIMAL RESIDUAL DISEASE USING NEXT-GENERATION SEQUENCING IN ACUTE MYELOID LEUKEMIA AND MYELODYSPLASTIC SYNDROMES

Phan Thị Xinh
Bệnh viện Truyền máu Huyết học.

Acute Myeloid Leukemia (AML) and Myelodysplastic Syndromes (MDS) are two myeloid malignancies characterized by complex disease progression and a high risk of relapse, even after intensive therapies and allogeneic hematopoietic stem cell transplantation (allo-HSCT). The assessment of Minimal Residual Disease (MRD) has become an essential tool for predicting relapse risk and guiding subsequent therapeutic decisions. Among current techniques, Next-Generation Sequencing (NGS) enables the detection of persistent gene mutations initially identified at diagnosis with high sensitivity, particularly post-transplantation, thereby enhancing the precision of MRD evaluation.

In AML patients, multiple studies have demonstrated that the persistence of genetic mutations identified and monitored via NGS before and after allo-HSCT is strongly associated with an increased risk of relapse and reduced overall survival (OS). Notably, MRD positivity at a month post-transplantation has emerged as a significant adverse prognostic marker, correlating with a higher relapse rate and lower OS, even among patients who were MRD-negative before transplant. In MDS, residual genetic mutations with a variant allele frequency (VAF) ≥ 0.5% at a month post-transplantation have been linked to disease progression and decreased progression-free survival (PFS). Furthermore, serial MRD monitoring using NGS has demonstrated the ability to detect molecular relapse earlier than clinical relapse, thus providing an opportunity for preemptive therapeutic intervention.

Recent studies also suggest that achieving “NGS-based MRD negativity” - the absence of detectable target mutations following treatment - has favorable prognostic implications, particularly in patients with MDS and secondary AML harboring TP53 gene mutations. Consequently, MRD monitoring using NGS is increasingly recognized as an indispensable component of AML and MDS management strategies, and it holds promise for early interventional treatment protocols in the near future.

Multidisciplinary Session 1-Laboratory

The predictive value of T-cell chimerism for disease relapse after allogeneic hematopoietic stem cell transplantation

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Introduction:
Chimerism is closely correlated with disease relapse after allogeneic hematopoietic stem cell transplantation (allo-HSCT). However, chimerism rate is dynamic changes, and the sensitivity of different chimerism requires further research.
Methods:
To investigate the predictive value of distinct chimerism for relapse, we measured bone marrow (BM), peripheral blood (PB), and T-cell (isolated from BM) chimerism in 178 patients after allo-HSCT.
Results:
Receiver operating characteristic (ROC) curve showed that T-cell chimerism was more suitable to predict relapse after allo-HSCT compared with PB and BM chimerism. The cutoff value of T-cell chimerism for predicting relapse was 99.45%. Leukemia and myelodysplastic syndrome (MDS) relapse patients' T-cell chimerism was a gradual decline from 2 months to 9 months after allo-HSCT. Higher risk of relapse and death within 1 year after allo-HSCT. The T-cell chimerism rates in remission and relapse patients were 99.43% and 94.28% at 3 months after allo-HSCT (P = 0.009), 99.31% and 95.27% at 6 months after allo-HSCT (P = 0.013), and 99.26% and 91.32% at 9 months after allo-HSCT (P = 0.024), respectively. There was a significant difference (P = 0.036) for T-cell chimerism between early relapse (relapse within 9 months after allo-HSCT) and late relapse (relapse after 9 months after allo-HSCT) at 2 months after allo-HSCT. Every 1% increase in T-cell chimerism, the hazard ratio for disease relapse was 0.967 (95% CI: 0.948–0.987, P<0.001).
Discussion:
We recommend constant monitoring T-cell chimerism at 2, 3, 6, and 9 months after allo-HSCT to predict relapse.

MULTIDISCIPLINARY SESSION 2

UNRELATED DONOR REGISTRY

Multidisciplinary Session 2-Unrelated donor registry

Enhancing Donor Provision Support in the Japan Marrow Donor Program through Behavioral and Digital Strategies

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In Japan, over 1,000 unrelated bone marrow and peripheral blood stem cell transplantations are performed annually through the Japan Marrow Donor Program (JMDP). Although more than 550,000 individuals are registered as potential donors, 60% are aged between 40 and 54. While younger donors are less likely to withdraw due to health reasons, previous studies have shown that many cancel coordination because of work obligations or lack of family consent. Therefore, reducing donor-driven cancellations remains a critical issue. To strengthen donor provision and reduce cancellations, we implemented a multi-faceted approach that combines behavioral economics, digital tools, and public engagement. To raise awareness of the donor leave system, we distributed 30-second and 5-minute videos through social media and the JMDP website, leading to an increase in annual company inquiries. We conducted a large-scale survey among 20–39-year-old donors (approximately 24,000 JMDP LINE subscribers), based on interviews with donors and their families, to better understand long-term motivation and the influence of family support. An international survey conducted with WMDA partners confirmed the widespread adoption of oral swab-based HLA typing and online registration. This supported Japan's transition toward more convenient and accessible donor registration methods. We also conducted a randomized controlled trial using behavioral economics-informed messaging during early stage of coordination. Messages emphasizing the rarity of HLA compatibility significantly increased confirmatory testing rates, particularly among male donors and those contacted repeatedly. In conclusion, our study demonstrates that a combined strategy—promoting donor leave, supporting family understanding, adopting online tools, and applying behavioral insights—can strengthen the donor provision system and ensure a more sustainable and younger donor pool within the JMDP. These strategies may serve as a model for donor programs across the Asia-Pacific region.

Multidisciplinary Session 2-Unrelated donor registry

Donor Selection in Allogeneic stem cell transplantation (SCT): role of unrelated donor registries

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Allogeneic stem cell transplantation (SCT) is a curative treatment for many life-threatening blood disorders. Donor selection for SCT traditionally focussed on minimising alloreactivity between immune systems of donor and recipient, with matching HLA-A, B, C and DRB1 (8/8 match) contributing most to long term survival. However, depending on patient age and ethnicity, <50% of recipients had available matched related donors (MRD), promoting the development of unrelated donor registries to expand donor access. More than 40million donors are now listed on registries world-wide, and depending on ethnicity, matched unrelated donors (MUD) are available to 29%-79% of recipients. Over recent years, outcomes for SCT utilizing non-HLA matched donors (alternative donors), has improved dramatically, largely due to use of novel graft versus host disease (GVHD) prophylaxis strategies, especially post-transplant cyclophosphamide (PTC). Alternative donor sources now include mis-matched unrelated donors (MMUD), related haplo-identical donors (haplo) and umbilical cord blood (UCB). Between HLA-matched and alternative donor sources, patients now have a >90% probability of finding a suitable donor match. In this setting, an increased emphasis on non-HLA factors in donor choice is emerging, including donor age and SCT timing. These developments have implications for donor search strategies (sequential versus concurrent) and registry procedures. A summary of current donor selection recommendations and the impact of these pathways in the Australian setting will be discussed.

MULTIDISCIPLINARY SESSION 3

QUALITY MANAGEMENT FACT-JACIE/ANALYZING AND REPORTING OUTCOMES

Multidisciplinary Session 3-Quality Management/FACT-JACIE/Analyzing and reporting outcomes

Quality management: FACT/JACIE Standards

Mickey BC KOH
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Quality Management is now fully embedded into haematopoietic stem cell transplant (HSCT)programs and is an essential element to ensure safety and consistency. This systematic approach is critical to good patient outcomes. The development of quality management has also seen the corresponding adoption of various accreditation schemes which includes JACIE, FACT and AABB. These accreditation pathways are often now embedded into transplant programs and in many countries are an essential criteria for licensing and approval by the health ministries. In addition, JACIE and FACT accreditation have become even more relevant as it has been used by commercial CAR-T as a benchmarking tool for commissioning and delivery of novel cell and gene therapies like CAR-T and genetic based therapies for curing haemoglobinopathies.
The development of such accreditation programs has been initiated in USA and Europe and linked to EBMT and ASTCT. As transplant programs are being initiated and developed worldwide, it has become evident that the journey to achieve accreditation will be vastly differing worldwide for centres, regions and countries. This is clearly a critical point that will need addressing urgently for HSCT and cellular therapy to move to the next phase worldwide. This issue has also been identified very early by APBMT who are doing excellent work into a step wise approach to such international accreditation. Other regional transplant organisations like the Latin American Bone Marrow Transplant (LABMT) have also started to look critically into this issue.
All of this will be discussed during the presentation.

Multidisciplinary Session 3-Quality Management/FACT-JACIE/Analyzing and reporting outcomes

Data Management of HCT: Experience and Reflections

CHEN Jia
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Robust data management is increasingly critical in the field of hematopoietic cell transplantation (HCT) due to its rapid procedural growth. Influential international data registries include EBMT in Europe and the US-based CIBMTR. In recent decades, Professor Huang's team is leading the data registry in China, that report the outlines of HCT status annually.

In Suzhou, an integrated data registry model merges a clinical database with a biobank. The structure includes the clinical database, the biobank, and a Quality Control Laboratory, covering full-process management of clinical data, sample collection, quality control, and utilization. This system is designed to support a range of functions, including data mining for clinical research and enabling translational research collaborations. The benefits of the data registry include the ability to timely depict clinical status, analyze resource utilization for departmental management, and facilitate patient follow-up.

European HCT data provides valuable benchmarks for follow-up rates and data completeness. In China, The China Portal Hypertension Alliance's (CHESS) Chronic Progressive Liver Disease Digital Management Platform (CDM) serves as an exemplary model for an advanced, multi-center system.

In the field of hematology, the future direction is smart data management. The platforms empower clinicians and researchers with advanced tools, such as intuitive graphical interfaces. Furthermore, they enable data visualization, transforming complex longitudinal data into dynamic graphic surveillance for chronic conditions like cGVHD through patient timelines, treatment-response charts, and multi-dimensional pathway diagrams. The ultimate goal is to move toward a "trinity" of data management, which involves the deep integration of clinical data, biological resources, and omics analysis within a unified framework. This integrated approach is essential for supporting complex real-world studies, enhancing research efficiency, and ultimately improving long-term outcomes for HCT patients.

Multidisciplinary Session 3-Quality Management/FACT-JACIE/Analyzing and reporting outcomes

Advancing HCT Research: Strategies for Analyzing Outcomes and Promoting Registry Studies

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Real-world data (RWD) encompasses information regarding patients' experiences, disease courses, and health intervention effects collected outside the framework of randomized controlled trials. RWD originates from various sources, including patient registries, electronic health records, claims data, and patient-reported outcomes. While patient registries are categorized as RWD, they often require data entry, unlike data automatically generated in daily practice. Nevertheless, registries offer a research advantage by allowing for predefined survey items and definitions.

Promoting the utilization of RWD in drug development and research is a growing imperative. The initial step in leveraging RWD involves a thorough search and evaluation of potential data sources to determine if they are "fit-for-purpose". This assessment includes examining the attributes of RWD sources, such as the size and representativeness of the patient population, data collection methods, and the availability of key variables like exposures and outcomes, including potential confounders. Ensuring data quality and comparability is critical to produce valid research findings.

In the field of Hematopoietic Cell Transplantation (HCT), activity has been ongoing since the 1960s, involving the collection of individual patient outcome data following transplantation. These registries have provided essential insights into the current state of HCT practice and have significantly contributed to the advancement of transplantation techniques and patient care. To further enhance the impact, active promotion of registry studies and utilization of HCT registry data are necessary. In promoting registry studies, it is essential to attract HCT physicians with research questions that contribute advancing the field of HCT.

Furthermore, ensuring high data quality and robust statistical analysis is essential. This involves not only maintaining rigorous data management but also unifying the definitions of fundamental variables for statistical analyses. Such standardization

enhances the reliability of research findings derived from registry data, ultimately supporting continuous improvement in HCT therapies and patient outcomes.

NURSE SESSION 1

GENERAL SCT NURSING CARE

Nurse Session 1-General SCT nursing care

Practical experience of NP-nurse collaboration in early intervention for HSCT patients through a Rapid Response System

Aoi Kato
St. Marianna University Hospital, Japan

Hematopoietic stem cell transplantation (HSCT) is vital for hematologic diseases, but patient decline affects outcomes. Rapid Response System (RRS) helps improve survival. At our hospital, NP involvement in RRS enables teamwork and quick responses for HSCT patients.

Nurse Session 1-General SCT nursing care

Program to improve the quality of care and enhance the competency of stem cell transplant nurses at the Ho Chi Minh City Hematology and Blood Transfusion Hospital

Ngo Thi Xuan Thao
Blood Transfusion Hematology Hospital, Ho Chi Minh City, Vietnam

Transplant nursing plays an important role in the success of a patient's stem cell transplant.
In collaboration with the Vietnamese transplant nursing team, we have implemented a project to improve the quality of transplant care through enhancing the capacity and knowledge of transplant nurses through the following activities:

- Participating in training sessions with the guidance of foreign experts, sharing experiences between other transplant centers
- Participating in conferences and seminars
- Develop a training module for HSCT nurses in our hospital
- Implementing training programs as well as assessing knowledge and capacity for transplant nurses

After 3 years of implementation, we have achieved certain results:

- Building a training module for transplant nurses at hospitals and in Ho Chi Minh City
- Improving knowledge and professionalism for transplant nurses

NURSE SESSION 2

LONG-TERM FOLLOW-UP CARE

Nurse Session 2-Long-term follow-up care

Vaccination following hematopoietic stem cell transplant

Hisayo Doi
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Patients who have undergone hematopoietic stem cell transplantation (HSCT) have weakened immune systems and are more susceptible to various infections. Once an infection occurs, treatment is often challenging. Additionally, after transplantation, the immunity acquired through natural infection or prior vaccination declines or disappears. Therefore, vaccination is recommended to help prevent disease onset or reduce symptom severity.
However, in Japan, post-transplant vaccination is not yet widely implemented, and our hospital was also not providing adequate vaccination. The reasons identified include: (1) complexity of the vaccination schedule, (2) lack of knowledge, and (3) the fact that it is not covered by insurance and requires out-of-pocket payment.
To address these issues, we reviewed recommendations from both domestic and international guidelines and discussed with BMT (Blood and Marrow Transplant) physicians, ID (infectious disease) specialists, LTFU (Long-Term Follow-Up) nurses, and the medical administration department to establish a vaccination system for post-HSCT patients at our hospital.
Based on these guidelines, we first clarified the types of vaccines, the recommended schedule, and the associated costs. When a patient becomes eligible for vaccination, the BMT physician—who is the primary doctor of the patient—assesses the patient's condition, including GVHD (Graft-versus-Host Disease) and overall health status, and consults with the ID department. At that point, the BMT physician and LTFU nurse explain the necessity and cost of the vaccinations to the patient and obtain their consent. The ID department, which is responsible for administering the vaccines, then handles the scheduling, provides explanations to the patient, and manages follow-up care.
In this presentation, I will introduce the vaccination system we have established at our hospital and its current implementation status, as well as provide an overview of the current state of secondary cancer prevention.

Nurse Session 2-Long-term follow-up care

Long-Term Care Model for Allogenic Hematopoietic Stem Cell Transplantation Patients via HSCT Platform

Chiang, Meng-Kuan
Koo Foundation Sun Yat-Sen Cancer Center

With improved survival after hematopoietic stem cell transplantation (HSCT), long-term survivorship care has become increasingly essential. However, comprehensive post-transplant support remains limited. At National Taiwan University Hospital, a baseline review of allo-HSCT patient outcomes revealed significant challenges: over 70% of patients developed graft-versus-host disease (GvHD) within the first year, with more than 49% of cases occurring within the first three months. Notably, eye and oral GvHD were frequently under-recognized until later stages, delaying interventions and impacting quality of life.

In response, a structured, case manager-led care model was implemented, incorporating scheduled follow-ups at 1 week, 3 months, 6 months, and 1–2 years post-transplant. This model emphasizes GvHD screening, infection prevention, functional assessment, and patient empowerment via mobile contact lines, a GvHD handbook, and an HSCT platform for self-monitoring. Preliminary data demonstrated improved early detection and care coordination, particularly for ocular GvHD.

However, retrospective evaluation of current practice revealed a key limitation—while patient-reported outcomes (PROs) were routinely collected, mechanisms for automated analysis and timely clinical response remained underdeveloped. This gap often led to missed opportunities for early intervention. In light of this, the next phase of development aims to build an innovative survivorship platform integrating PROs with AI-driven analytics. Through real-time symptom tracking and automated triage, nurse case managers will lead individualized, responsive care. Despite challenges such as data standardization and workflow integration, this model represents a scalable, intelligent, nurse-led solution to optimize long-term HSCT outcomes.

Nurse Session 2-Long-term follow-up care

Long-Term Follow-Up Care After Hematopoietic Stem Cell Transplantation

Min-Ji Kwak
National University of Hospital, Seoul, Korea

Long-term follow-up after hematopoietic stem cell transplantation is essential for optimal patient care. Regular monitoring enables early detection and management of chronic GVHD and infections, which are major causes of morbidity and mortality. Multidisciplinary collaboration and individualized surveillance protocols help prevent complications, support timely interventions, and improve long-term outcomes and quality of life for transplant recipients.

NURSE SESSION 3

CAR-T CELL THERAPY

Nurse Session 3-CAR-T Cell Therapy

Key Nursing Points for CAR-T Cell Therapy - Insights from Our Experience in Japan –

Mayumi Sumita
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In the mid of the 19th century, there were no curative therapies for hematological malignancies. Despite remarkable progress of medical treatment, there are still many relapsed/refractory (r/r) patients. In 2019, the Japanese Ministry of Health, Labour and Welfare (MHLW) approved only for the treatment of patients with r/r B-cell acute lymphoblastic leukemia and r/r large B-cell lymphoma (LBCL). As of 2025, four types of CAR-T cells (tisagenlecleucel, axicabtagene ciloleucel, lisocabtagene maraleucel, and idecabtagene vicleucel) are available for clinical use in Japan, and their application has been expanded to be applicable to r/r follicular lymphoma and r/r multiple myeloma. Our hospital was the first facility in Japan to perform CAR-T cell therapy for LBCL in 2016, and we have provided approximately 150 patients to date.

Cytokine Release Syndrome (CRS) and Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS) are representative adverse events that require during hospitalization. The characteristic symptom of CRS is fever, which can worsen to respiratory failure and hypotension in severe cases. The incidence varies depending on the disease and type of CAR-T cells, but CRS is observed in 45–93% of cases, with Grade 3 or higher severe CRS occurring in 0–50% of cases. ICANS may occur during or more commonly after CRS symptoms, and may manifest as delirium, encephalopathy, aphasia, difficulty concentrating, agitation, tremor, seizures, and cerebral edema. ICANS was observed in 5–65% of cases, with severe ICANS (Grade 3 or higher) occurring in 1–34% of cases. Since it can be fatal, appropriate management is required.

In this session, I will provide an overview of nursing care for these adverse events following CAR-T cell infusion. In addition, I will present actual case that we have experienced to have a better understanding.

Nurse Session 3-CAR-T Cell Therapy

Adoptive Cellular Therapy post HSCT

Pham Thi Ngoc Anh
SingHealth Academic Medical Centre, Singapore

Adoptive cellular therapy has emerged as a significant advancement in managing post-transplant complications, including viral reactivation, mixed chimerism, and immune reconstitution. This therapeutic approach demonstrates marked advantages over conventional treatments, offering improved patient outcomes. As frontline healthcare providers, nurses play a pivotal role in care delivery, patient monitoring, and family education. Therefore, continuous nursing education and competency development in cellular therapy are essential to ensure optimal patient care and treatment success.

Nurse Session 3-CAR-T Cell Therapy

CAR-T cell therapy experience-Taiwan

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Hematology & Cellular Therapeutics Center, National Taiwan University Hospital, Taipei, Taiwan

The development and implementation of CAR-T cell therapy in Taiwan have significantly advanced the treatment landscape for hematologic malignancies. Since its introduction, 8 major medical centers such as National Taiwan University Hospital have pioneered the clinical application of CAR-T therapy since 2022 September, particularly for patients with relapsed or refractory B-cell acute lymphoblastic leukemia (ALL) and diffuse large B-cell lymphoma (DLBCL). The Taiwan Food and Drug Administration (TFDA) has approved commercial CAR-T products like Kymriah (tisagenlecleucel), and Taiwan's National Health Insurance began covering CAR-T therapy in 2023, improving patient access to this innovative treatment.

Clinical teams in Taiwan have developed comprehensive protocols to manage the entire CAR-T process, from leukapheresis and cell manufacturing to infusion and post- treatment monitoring, ensuring patient safety and optimal outcomes. Early experiences demonstrate promising remission rates and manageable side effects, reflecting the therapy's potential to transform care for patients with limited treatment options. Additionally, domestic biotechnology companies are actively conducting clinical trials to develop local CAR-T products, aiming to expand indications and enhance therapeutic efficacy. Overall, Taiwan's CAR-T cell therapy experience highlights a successful integration of cutting-edge immunotherapy into clinical practice, offering new hope for patients battling hematologic cancers.

ORAL PRESENTATION

Oral Presentation

Allogeneic Hematopoietic Stem Cell Transplantation as Consolidation Therapy After BCMA CAR-T Cell Treatment Significantly Improves Overall Survival in Patients with Extramedullary Plasmacytoma and Plasma Cell Leukemia

Lixia ma
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Background:
Extramedullary disease (EMD) and plasma cell leukemia (PCL) represent aggressive manifestations of multiple myeloma (MM). Previous studies have shown that these conditions have significantly worse prognoses compared to MM patients without soft tissue plasmacytomas. While B-cell maturation antigen (BCMA)-targeted chimeric antigen receptor (CAR) T-cell therapy has demonstrated promising efficacy in relapsed/refractory (R/R) MM, clinical data regarding its application in R/R EMD and PCL remain limited.

Aim:
This study retrospectively analyzed the clinical efficacy and survival outcomes of EMD and PCL patients treated with BCMA-CAR-T therapy at our center.

Methods:
This single-center retrospective study included 39 patients with R/R MM who received BCMA-CAR-T therapy at Beijing GoBroad Hospital between March 2019 and November 2024. Among them, 26 had extramedullary plasmacytoma (EMD), and 13 had plasma cell leukemia (PCL), including 8 with both PCL and EMD. The cohort included 24 males (61.5%), with a median age of 59 years (range: 35–82). Genetic high-risk factors (including 1q21+, t(4;14), t(14;16), t(14;20), 17p-, and p53 mutations) were identified in 23 patients (59.0%). A total of 20 patients (51.3%) had an ECOG score ≥2. The median number of prior treatment lines was 5 (range: 2–9). Triple-class refractory disease was observed in 29 patients (74.4%), while 10 patients (25.6%) were refractory to five classes of drugs. Additionally, 20 patients (51.2%) had undergone prior autologous stem cell transplantation, and 9 (23.0%) had received localized radiotherapy. Baseline patient characteristics are

summarized in Table 1. (*Table 1, attached at the time of submission, was deleted due to space limitations. —BCT Editorial Office*)

Results:

(1) Overall Safety and Efficacy

Among all patients, 71.7% (28/39) received tumor debulking therapy, including the DECP regimen and localized radiotherapy. Tumor debulking efficacy was evaluable in 15 patients, yielding an overall response rate (ORR) of 53.3% (8/15), including 2 with very good partial response (VGPR) and 6 with partial response (PR). The median BCMA CAR-T infusion dose was 0.56×10^6/kg (range: 0.011–5.9), and the median CAR-T expansion peak was 10.5×10^6/L (range: 0.129–1710), occurring at a median of day 14 post-infusion (range: 4–45). Cytokine release syndrome (CRS) occurred in 33/39 (84.6%) patients, with 5 (12.8%) experiencing grade ≥3 CRS. Immune effector cell-associated neurotoxicity syndrome (ICANS) was observed in 7/39 (17.9%) patients, all of whom experienced grade ≥3 ICANS.

The best ORR after CAR-T cell therapy was 64.1% (25/39), with a complete response rate (CRR) of 41.0% (16/39). The median time to best response was 60 days (range: 21–105). At a median follow-up of 20.35 months (range: 0.1–60.94), the median overall survival (OS) was 14.6 months, with a 1-year OS rate of 54.57%. The median progression-free survival (PFS) was 5.62 months, with a 1-year PFS rate of 37.22%.

(2) Subgroup Analysis

A. Survival in EMD and PCL (or PCL+EMD) Patient

Among 26 EMD patients (66.6%) and 13 PCL/PCL+EMD patients (33.3%), the median OS was 12.43 months vs. 14.6 months, respectively, with 1-year OS rates of 53.65% vs. 53.85%. The median PFS was 4.87 months vs. 9.27 months, with 1-year PFS rates of 34.63% vs. 46.15%.

B. Survival Based on Response to CAR-T Therapy

Among patients who had an effective response to CAR-T therapy vs. those who did not, the median OS was 18.5 months vs. 2.54 months ($p<0.0001$), with 1-year OS rates of 76.77% vs. 14.29%. The median PFS was 14.6 months vs. 1.63 months ($p<0.0001$), with 1-year PFS rates of 61.92% vs. 0%.

C. Survival Based on Consolidation Therapy Post-CAR-T

Among the 25 patients who had an effective response to CAR-T therapy, 18 received consolidation therapy: 6 underwent allogeneic hematopoietic stem cell transplantation (allo-HSCT), while 12 received other consolidation therapies (including targeted therapy, chemotherapy, or localized radiotherapy). The median OS for the allo-HSCT group was not reached, while it was 15.52 months for the non-transplant consolidation group. The median PFS was 39.54 months vs. 9.27 months, respectively. The 1-year OS rates were 100% vs. 61.36% (p=0.0482), while the 1-year PFS rates were 66.67% vs. 48% (p=0.3621).

Conclusion:
BCMA-CAR-T cell therapy is an effective treatment strategy for EMD and PCL. The use of allo-HSCT as consolidation therapy after an effective CAR-T response significantly improves overall survival in these patients.

Oral Presentation

Clinical Outcomes of Locally Manufactured CD19 CAR-T Cell Therapy for Relapsed/Refractory B-ALL and B-NHL in Malaysia

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Background and Aim:
CD19-targeted CAR-T therapy has transformed treatment for relapsed/refractory (R/R) B-ALL and B-NHL, but access in Malaysia is limited due to high costs and absence of approved products. Plutonet Sdn Bhd developed a locally manufactured CD19 CAR-T product (PP CAR-T) to offer an affordable, accessible solution.

Methods and Patients:
Two open-label, single-arm Phase II trials were conducted at Pusat Terapi Sel, UKM (2019–2025; NCT03937544, NCT06698484). Of 73 enrolled patients, 30 patients (8 B-ALL and 22 B-NHL) received autologous PP CAR-T cells. Median follow-up was 12.5 months.

Manufacturing and Delivery:
Median time from apheresis to culture start was 61.5 days, culture duration was 14 days, and most patients received fresh infusions.

Safety and Tolerability:
CRS occurred in 75% of B-ALL (all grade I–II) and 46.7% of B-NHL patients (one fatal event). No ICANS cases were observed. Grade ≥3 cytopenias were common but manageable. Infections were infrequent and well-controlled.

Efficacy:
All B-ALL patients achieved complete remission (CR), with 87.5% MRD-negative. Sustained remissions were observed in 50% at 12–16.8 months. B-NHL patients showed an 81% overall response rate (ORR) and 58.8% CR one month post-infusion, with durable responses at 52.4% at 12 months.

Cell Expansion Kinetics:
Robust CAR-T expansion (peak CD19+ CAR-T >10%) correlated with early CR and better durability. Patients with poor expansion had higher relapse rates.

Conclusion and Future:

PP CAR-T therapy manufactured in Malaysia demonstrated comparable safety and efficacy to commercial products like Tisa-cel while addressing local barriers to access. Early referral and rapid T-cell collection are critical. Larger trials are needed to confirm these encouraging findings.

Oral Presentation

IL-17A and IL-18 as Key Predictors in a Novel Model for Severe aGVHD Post-Allogeneic Hematopoietic Stem Cell Transplantation

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Aims:
We aim to establish a method for predicting severe acute graft-versus-host (aGVHD) through analysis of peripheral blood inflammatory proteins in patients receiving allogeneic hematopoietic stem cell transplantation (allo-HCT).

Methods:
Peripheral blood plasma samples from 153 patients were prospectively collected on day 14 after allo-HCT. A total of 92 inflammatory proteins were quantitatively measured using the proximity extension assay. A method for predicting severe aGVHD after allo-HSCT was established by differential analysis and logistic regression model.

Results:
Within 100 days after allo-HCT, 33 out of 153 patients (21.6%) developed severe aGVHD. Samples for analysis were selected based on the severity of aGVHD after transplantation. A total of 88 samples were included: 33 from patients with severe aGVHD, 17 with grade 2, and 38 with grade 0–1 aGVHD. Among the 88 patients, 60 (68.2%) underwent haploidentical HSCT and 28 (31.8%) underwent matched sibling donor HSCT. The median time of aGVHD onset was 32 (IQR 25 - 45) days after transplantation. Compared with patients without severe aGVHD, the plasma levels of IL-17A, IL-18, IL-10, PD-L1, CXCL9, uPA, TNF-β, CD244, CXCL10, ADA, and IFN-γ were significantly upregulated in patients with severe aGVHD, and FGF-19 levels were significantly downregulated. These differentially expressed genes are significantly enriched in pathways including positive regulation of cytokine production, positive regulation of immune effector processes, type II interferon production, and cell migration. After applying Lasso regression analysis, IL-17A, IL-18, CD244, and FGF-19 were included in a logistic regression model. Logistic regression analysis revealed that IL-17A and IL-18 were significantly positively associated with severe aGVHD. Specifically, IL-17A had a coefficient of 0.7007 (P=0.007), and IL-18 had a coefficient of 0.6478 (P=0.019). CD244 showed a positive but non-significant association with a coefficient of 0.5065 (P=0.331). FGF-19 was negatively associated with severe aGVHD (coefficient = -0.5924, P = 0.079), suggesting a potential protective effect, although this did not reach statistical significance. The model predicted severe aGVHD with an area under the receiver operating characteristic curve (AUROC) of 0.800. Internal validation using the

bootstrap method yielded an AUROC of 0.784 (95% CI: 0.783–0.785) and a precision of 0.741 (95% CI: 0.739–0.744).

Conclusions:
IL-17A and IL-18 made major contributions to the logistic regression model for predicting aGVHD. With large-scale cohort studies and thorough validation, this model has the potential to become an effective clinical tool for predicting severe aGVHD.

Oral Presentation

Polatuzumab-based Chemotherapy Followed by CAR-T Therapy in Relapsed/Refractory Burkitt Lymphoma: A Real-World Analysis

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Background:
Burkitt lymphoma (BL) is a highly aggressive B-cell malignancy. Although most patients can achieve remission with frontline intensive chemotherapy regimens, those with relapsed or refractory (R/R) disease continue to face dismal outcomes, with a median overall survival often less than six months. Currently, therapeutic options for R/R BL remain extremely limited.

Aims:
To evaluate the response rate of polatuzumab vedotin–based chemotherapy in patients with relapsed or refractory Burkitt lymphoma (R/R BL), and to assess the long-term survival outcomes following sequential CAR-T cell therapy.

Methods:
This single-center retrospective study analyzed patients with relapsed or refractory Burkitt lymphoma (R/R BL) who received polatuzumab vedotin (Pola)-based chemotherapy at Beijing GoBroad Hospital between October 2020 and March 2025. Pola-based chemotherapy regimens included PBR (polatuzumab vedotin, bendamustine, rituximab) and PICE (polatuzumab vedotin, ifosfamide, carboplatin, etoposide).A total of 23 patients were included. Among them, 18 (78%) subsequently received CAR-T cell therapy following Pola-based treatment.Seventeen patients (74%) were male, with a median age of 36 years (range, 20–75). Fourteen patients (61%) had received ≥3 prior lines of therapy, and 21 (91%) had advanced-stage disease (Ann Arbor stage III–IV). Next-generation sequencing was performed in 18 patients (78%), focusing on key genetic alterations including TP53-mutation(14/18), ID3-mutation(8/18), and MYC-mutation(11/18). Three patients (13%) had failed prior autologous hematopoietic stem cell transplantation (ASCT), and 3 (13%) and 5 (22%) had received prior radiotherapy and CAR-T cell therapy, respectively.

Polatuzumab vedotin was administered at a dose of 1.8 mg/kg in combination with chemotherapy regimens tailored to individual patients. Eighteen of the 23 patients (78%) proceeded to CAR-T cell therapy following disease debulking with the Pola-based regimen.Efficacy was assessed using standard response criteria, including complete response (CR), partial response (PR), stable disease (SD), and progressive disease (PD). The objective response rate (ORR) was defined as the proportion of patients achieving CR or PR, and the disease control rate (DCR) as the proportion achieving CR, PR, or SD. Survival outcomes including overall survival (OS) were evaluated from the date of first Pola-based therapy to death or last follow-up, using Kaplan-Meier analysis.

Results:

Among the 23 patients with R/R BL treated with Pola-based chemotherapy, the overall response rate (ORR) was 26% (6/23), including one complete response (CR) and five partial responses (PR). Notably, the disease control rate (DCR) reached 74% (17/23), with an additional 11 patients achieving stable disease (SD). Although the objective response rate was limited, the high DCR suggests that Pola-based regimens were effective in stabilizing disease, which facilitated subsequent CAR-T cell therapy in most patients.Polatuzumab-based chemotherapy resulted in frequent hematologic toxicity. Neutropenia occurred in 96% (22/23) of patients, all grade ≥3. Thrombocytopenia and anemia were observed in 91% (21/23) and 96% (22/23), respectively, with grade ≥3 events in 61% (14/23) for both.

Eighteen patients (78%) proceeded to CAR-T cell infusion following Pola treatment, with a median bridging time of 29 days (range: 6–131). Among CAR-T recipients, the best overall response rate was 67% (12/18). In the total cohort (N=23), the three-month ORR following CAR-T therapy was 35%, with a CR rate of 30%.

CAR-T-related toxicities were generally manageable. Cytokine release syndrome (CRS) occurred in 78% (14/18), with only one case of grade ≥3 severity. Immune effector cell-associated neurotoxicity syndrome (ICANS) occurred in 1 patient (6%). The median infused CAR-T cell dose was 1.6925×10^6/kg, with a median peak expansion on day 11 (range: 3–22), reaching 2,466.5 copies by PCR and 3.0×10^7/kg by flow cytometry.

With a median follow-up of 18.64 months, the overall cohort had a median overall survival (OS) of 6.31 months. The 6-month and 1-year OS rates were 50.6% and 32.1%, respectively. Among patients who did not achieve remission after CAR-T (N=6), the median OS was 5.455 months.

Summary/Conclusion:

Polatuzumab vedotin–based chemotherapy demonstrated limited efficacy as salvage treatment in relapsed/refractory Burkitt lymphoma, but showed potential as a bridging strategy to CAR-T cell therapy by achieving disease control in a substantial proportion of patients. Sequential CAR-T therapy was feasible and associated with encouraging response and survival outcomes in this high-risk population. These findings support the integration of Pola-based regimens into multi-modal treatment strategies for R/R BL and warrant further prospective validation.

Oral Presentation

The Impact of Letermovir Prophylaxis in Matched Sibling Donor Hematopoietic Stem Cell Transplantation: Selecting the Appropriate Population for Optimal Prophylactic Therapy

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Aims:
Letermovir (LTV) has been effectively used to prevent cytomegalovirus (CMV) reactivation in CMV-seropositive patients. Previous findings primarily involve haploidentical or cord blood transplantation, evidence on LTV's effects in matched sibling donor transplant remains limited. This study was conducted to evaluate the impact of LTV in matched sibling hematopoietic stem cell transplantation (HSCT) recipients.

Methods:
We retrospectively compared 72 matched sibling transplantation recipients who received LTV with 134 patients without LTV at the Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, between January 2022 and December 2023. Cumulative incidence of CMV- or EBV-related complications was evaluated using Gray's test, accounting for competing risks such as death, relapse, and additional hematopoietic stem cell transplantation. Independent risk factors for CMV viremia were identified through univariable analysis, with variables reaching $P<0.05$ included in a multivariable cox competing analysis. Propensity score matching (PSM) with the "optimal" method was used to balance baseline characteristics between groups. OS, DFS, GRFS and NRM were evaluated using the Kaplan-Meier method and compared with log-rank tests.

Results:
LTV significantly reduced 200-day CMV viremia incidence (5.6% vs. 33.3%, $P<0.001$) (Figure 1C). Multivariable analysis identified anti-thymocyte globulin (ATG) use (HR 2.92, 95%CI 1.48-5.78, $P=0.002$) and grade III-IV acute graft-versus-host disease (GVHD; HR 4.01, 95%CI 2.20-7.33, $P<0.001$) as independent risk factors for CMV viremia, while CMV serostatus did not significantly affect CMV viremia ($P=0.448$). Stratification based on risk factors (ATG, grade III-IV aGVHD) showed

that LTV had a more significant effect on high-risk patients compared to low-risk patients (Figure 1A). Also, LTV prophylaxis was associated with increased Epstein-Barr virus (EBV) viremia (Figure 1E) and higher CD20 monoclonal antibody utilization. Long-term survival (1-year OS, DFS, GRFS and NRM) remained similar between both groups (Figure 2A, B, C, D). The findings were validated in a second retrospective cohort from our center (Figure 1B, D, F).

Conclusions:

This study demonstrates that LTV prophylaxis significantly reduces 200-day CMV viremia in matched sibling HSCT recipients, particularly in high-risk patients with ATG exposure or grade III-IV aGVHD. However, LTV use was associated with an increased risk of EBV-related complications, underscoring the need for careful patient selection. Notably, in our study population—where CMV seropositivity rates are substantially higher than those in Western cohorts—CMV serostatus alone did not predict reactivation risk completely, which may preclude its use as a standalone criterion for LTV prophylaxis. These findings support risk-stratified CMV prevention over unselected serostatus-based strategies. Further studies are warranted to balance CMV protection against EBV reactivation risks.

Oral Presentation

Veno-Occlusive Disease in Hematopoietic Stem-Cell-transplant: A Multi-Year Review of Patterns, Treatment Challenges, and Outcomes

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Aims:
Hepatic Veno-Occlusive Disease (VOD) is a life-threatening complication following hematopoietic stem cell transplantation (HSCT), causing significant morbidity and mortality, particularly in allogeneic transplants. This study analyses the incidence, clinical characteristics, and outcomes of patients who developed VOD post-HSCT over 14 years, with a focus on risk factors and treatment limitations.

Methods:
Patients undergoing hematopoietic stem cell transplants from January 2011 to March 2025 were included in the study. Veno-occlusive disease (VOD) was identified using the Seattle criteria(1). Mild cases of VOD were those where the illness resolved on its own or with minimal intervention (other than restarting ursodiol). Moderate VOD involved treatment like Defibrotide, diuretics for fluid retention, or pain relief for hepatomegaly, leading to eventual recovery. Severe VOD was marked by multiorgan dysfunction, persistent liver-related symptoms, or fatal outcomes. Unfortunately, data limitations prevented an accurate calculation of the latest EBMT severity scores. Details of patients treated with Defibrotide for VOD were recorded throughout the study. Baseline demographic and epidemiology were analysed using descriptive statistics.

Results:
Between 2011 and 2025, 868 hematopoietic transplants (491 allogeneic and 377 autologous) were performed for various indications. Twenty-two patients (4.48%) developed hepatic Veno-occlusive disease. The baseline characteristics of these patients are summarised in Table 1. Median age of the patients was 33 years (range: 5-59 years). Most patients (n=21, 95.5%) developed VOD after allogeneic stem cell transplants, while one patient developed late-onset VOD after an autologous stem cell transplant for relapsed APML. Five patients (22.7%) had received Inotuzumab for a median of 2 cycles (range: 1-4) prior to transplant. All patients received myeloablative (MAC) or reduced toxicity ablative (RTC) conditioning regimens (19

MAC and 4 RTC). All received a PBSC graft with a median cell dose of 4.40 x 10^6/Kg (2.85-7.63).

Fourteen (63.6%) achieved neutrophil engraftment at a median of 14 days, while eight (36.4%) died before neutrophil engraftment. Five patients (22.7%) achieved platelet engraftment (one at 8 days, one at 9 days, and three at 13 days) before developing VOD, while three patients experienced delayed platelet engraftment beyond 28 days (30 days for two patients and 36 days for one patient) after developing VOD. Fourteen patients died before achieving platelet engraftment. Eight (36.4%) patients developed graft versus host disease (6 had gut GVHD, 3 had skin GVHD).

Moderate and severe VOD were seen in 7 and 14 patients respectively. 2 patients were treated for severe VOD with low dose Defibrotide. However, both these patients succumbed to the ailment. VOD was considered a contributing factor to mortality in all 14 patients who died, with concomitant severe infection in two (14.28%) and steroid-refractory graft-versus-host disease in two (14.28%) patients.

Conclusion:
VOD remains a severe post-transplant complication, predominantly affecting allogeneic HSCT recipients. Limited access to Defibrotide and high mortality rates underscore the urgent need for improved preventive and therapeutic strategies. Understanding patient risk profiles and optimizing conditioning regimens may help mitigate VOD incidence. Further research is essential to enhance treatment approaches and improve survival outcomes.

Oral Presentation

Fifteen year follow up of haploidentical HSCT for children with IEI - TCR-αβ/CD 19 depleted or T replete graft with post-transplant cyclophosphamide in children undergoing haploidentical hematopoietic stem cell transplant for inborn error of immunity

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Background & Aim:
A haploidentical-related donor is readily available for children with inborn errors of immunity (IEI) requiring hematopoietic stem cell transplantation (HSCT) as alternative donors for those with no matched family donors, with TCRαβ/CD19-depletion and post-transplant cyclophosphamide (PTCY) being the predominant techniques for T-cell depletion. Our study aimed to analyze the outcomes of TCRαβ/CD19 depleted graft and T-replete graft with PTCY in children with IEIs.

Methods:
We conducted a retrospective analysis of the children with IEI who underwent haploidentical donor HSCT at our center between April 2009 to September 2024 over a 15-year period. We collected data on the underlying diagnosis, donor source, conditioning, engraftment, graft versus host disease (GVHD), viral reactivation, and survival from chart reviews and performed statistical analysis using SPSS software. The study was approved by our Institutional Ethics Committee.

Results:
From April 2009 to September 2024, we performed HSCT for 230 children with inborn errors of immunity, of which 105(45%) children had a haploidentical HSCT. We used a TCR deplete technique in 65(62%) children while T cell replete transplant was done in 40(38%) children.

Engraftment rates were similar in both the groups. The rates of acute GVHD and chronic GVHD between TCRαβ/CD19-depletion vs PTCY were 5(7.5%) vs 6(15%) and 9(14%) vs 7(17%) respectively. Viral reactivation rates were 65% vs 45% respectively in TCRαβ/CD19-depleted vs PTCY cohorts. The overall survival in the TCRαβ/CD19-depleted cohort vs PTCY were 66% vs 55%.

The overall survival in this cohort was 62%, with most of the mortality secondary to viral infections. Mortality was higher among those with poor immune reconstitution. The individual variables under study have been listed in the table as below.

Conclusion:

The survival was superior in TCRαβ/CD19-depletion compared to PTCY with viral reactivation rated been extremely high in the former group. The promise of early engraftment and low morbidity during HSCT and low rates of graft versus host disease was offset by persistent viraemia. We need early diagnosis of IEI by raising awareness with timely interventions improves the overall outcomes. Letermovir prophylaxis and memory cell addback to improve immune reconstitution and viral specific T cell infusions will result in over 90% survival as seen in data from high income countries. Despite higher cost, the way forward for children with IEI would be haploidentical HSCT using TCRαβ/CD19-depletion.

Oral Presentation

Reduced- Versus Standard-Dose Melphalan as Single-Agent Conditioning Regimen in Autologous HSCT for Multiple Myeloma: A Systematic Review and Meta-Analysis

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Background: The optimal conditioning dose of melphalan for autologous hematopoietic stem cell transplantation (AHSCT) in multiple myeloma remains a subject of debate. While prior studies have presented conflicting evidence on whether standard-dose melphalan (SDM, 200 mg/m^2) provides a statistically significant survival advantage over reduced-dose melphalan (RDM, 140 mg/m^2), further investigation is necessary to clarify this.

Aim: This systematic review and meta-analysis aimed to consolidate existing studies and assess whether standard-dose melphalan confers a significant benefit in terms of overall survival (OS) and progression-free survival (PFS) compared to reduced-dose melphalan, while also evaluating secondary outcomes non-relapse mortality (NRM), transplant-related mortality (TRM), and engraftment time (ET).

Methods: A comprehensive literature search across four electronic databases (i.e., The Cochrane, Clinicaltrials.gov, PubMed, Google Scholar) identified six eligible studies published in the last 10 years, with a pooled sample size of 2,827 patients. The meta-analysis was conducted for the primary outcomes (OS and PFS) using hazard ratios (HRs) and their 95% confidence intervals (CIs), with effect sizes calculated based on reported p-values, population sizes, and Cohen's framework for effect size estimation. A fixed-effects model was employed, assuming relative homogeneity of effect sizes across studies.

Results: For overall survival, no significant difference was seen between standard-dose and reduced-dose melphalan (95% CI: -0.0379 to 0.1687, p = 0.1647). Similarly, no significant difference in PFS was observed (95% CI: -0.023 to 0.0782, p = 0.2248). The effect sizes for OS (0.0654) and PFS (0.0274) were small, indicating little clinical relevance despite trends to advantages in OS (7.6 months) and PFS (4.5 months) for the standard-dose group. The systematic review of secondary outcomes (NRM, TRM,

and ET) demonstrated no significant differences between the two dose groups. Heterogeneity for primary outcomes was low (I^2 = 9.73%).

Conclusions: Although standard-dose melphalan shows better survival trends, statistical analysis shows no significant difference in overall or progression-free survival. The small effect sizes also suggest these differences are not clinically meaningful. Due to lack of clear survival benefit between SDM and RDM, choice should be based on other important patient factors like risk of side effects and ability to tolerate treatment.

Oral Presentation

Incidence and Risk Factors for Zoster Reactivation Post Haematopoietic Stem Cell Transplant in Singapore General Hospital

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Introduction: Reactivation of zoster post haematopoietic stem cell transplant (HSCT) can lead to serious complications, including disseminated infection and even death. Although antiviral prophylaxis is typically administered for one year following transplantation, zoster reactivation may still occur up to three years later. with a high incidence noted up to three years post-HSCT. There is also no consensus regarding whether antiviral prophylaxis should be continued beyond one year in post-HSCT patients without risk factors for reactivation, due to concerns over increased costs and potential side effects.

Aims: Given the lack of local data, this study aims to investigate the local incidence of zoster reactivation and identify the risk factors associated with late-onset zoster reactivation among post-transplant patients.

Methods: We conducted a retrospective cohort study involving 631 patients at a tertiary hospital in Singapore from January 1, 2012 to December 31, 2022. Additionally, a nested case-control study with 295 patients was undertaken to identify risk factors for zoster reactivation in our allogeneic transplant patients. Descriptive statistics were used to summarize baseline demographic data and clinical characteristics while cumulative incidence curves were employed to estimate the time to zoster reactivation, with relapse, non-tandem second transplant, and death considered as competing risks. Univariable and multivariable logistic regression analyses were performed to assess the association between risk factors and zoster reactivation in patients following allogeneic HSCT.

Results: At 10 years post-transplant, the cumulative incidence of zoster reactivation was 29% in allogeneic transplant recipients, significantly higher than the 19% observed in those who underwent autologous transplantation. A peak in zoster reactivation occurred two years after transplantation, potentially indicative of rebound reactivation following the cessation of antiviral prophylaxis. Multivariable statistical analysis revealed that the continued use of immunosuppressants after stopping antiviral prophylaxis (adjusted OR: 4.1; 95% CI: 1.2 – 14.7) was the sole

risk factor linked to an increased likelihood of zoster reactivation in post allogeneic transplant patients.

Conclusion: Patients undergoing allogeneic transplantation are at increased odds for zoster reactivation. To effectively minimize long-term zoster reactivation in our post-transplant survivors, consideration of extended antiviral prophylaxis may be warranted for patients with continued immunosuppression after one year. Future research should focus on evaluating the effectiveness of recombinant zoster vaccination in this population.

Oral Presentation

Minute FLT3-ITD Clones Detected at Day +30 After Allo-HSCT Identify High Risk of Relapse and Guide Subsequent Maintenance Therapy in AML Patients: A NICHE Cohort Study

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Aims:
Allogeneic hematopoietic stem cell transplantation (allo-HSCT) improves the prognosis of acute myeloid leukemia (AML) patients harboring *FLT3*-internal tandem duplication (*FLT3-ITD*) mutations. However, 15%–35% of patients still experience post-transplant relapse, underscoring the need for high sensitive early detection methods to guide timely interventions. Currently, most centers rely on capillary electrophoresis-based fragment analysis for *FLT3-ITD* monitoring, which offers limited sensitivity (10^{-2}). As a result, *FLT3-ITD* positivity is often detected when minimal residual disease (MRD) or morphological relapse has occurred. While MRD status at day +30 post-HSCT has been recognized as prognostically significant, the presence and clinical implications of minimal *FLT3-ITD* clones at this early time point remain unclear. Leveraging the NICHE-BMT cohort, we investigated whether high-sensitive next-generation sequencing (NGS; sensitivity 10^{-6}) for *FLT3-ITD* detection at day +30 could identify patients at high risk of relapse and inform decisions on post-transplant maintenance therapy.

Methods:
A total of 2,711 patients with malignant hematologic diseases who underwent HSCT between September 2017 and September 2024 were enrolled from the NICHE-BMT cohort. Adult patients with an initial diagnosis of AML with *FLT3-ITD* mutations who received allo-HSCT were included. Patients diagnosed with secondary AML, Acute promyelocytic leukemia, or those who had not achieved morphological completely remission prior to transplantation were excluded. Ultimately, 136 eligible patients were included, and their day +30 post-transplantation samples were retrospectively analyzed for minimal *FLT3-ITD* mutations using NGS.

Results:
The median age at transplantation among 136 AML patients was 39 years (IQR, 29–49), with a median follow-up of 24.9 months (IQR, 14.5–37.6). All patients were

FLT3-ITD negative at day +30 by capillary electrophoresis-based fragment analysis. However, high-sensitive NGS detected minute *FLT3-ITD* clones in 27.2% (n = 37) of patients. The cumulative incidence of post-transplant MRD positivity by flow cytometry was significantly higher in *FLT3-ITD*–positive patients compared to negatives (40.3% [95% CI, 38.8–41.7] vs. 18.8% [95% CI, 18.4–19.1], *P* = 0.001) (Figure 1A). *FLT3-ITD*–positive patients showed a higher 2-year cumulative incidence of relapse (CIR) (22.8% [95% CI, 21.8–23.9] vs. 15.2% [95% CI, 14.8–15.5], *P* = 0.233) (Figure 1B), and lower 2-year overall survival (OS) (70.9% [95% CI, 57.0–88.0] vs. 76.0% [95% CI, 67.3–85.8], *P* = 0.540) (Figure 1C). Regarding post-transplant maintenance therapy guided by *FLT3-ITD* status, we found that maintenance therapy significantly reduced 2-year CIR (8.3% [95% CI, 7.7–9.0] vs. 54.3% [95% CI, 47.8–60.8], *P* = 0.006) among positive patients (Figure 2A–C). In negative patients without pre-transplant high-risk factors (2017 ELN adverse risk, relapsed/refractory AML, or pre-transplant MRD/molecular positivity), maintenance therapy did not yield a survival benefit (2-year CIR: 6.3% [95% CI, 5.5–7.0] vs. 4.8% [95% CI, 4.3–5.2], *P* = 0.784; OS: 94.4% [95% CI, 84.4–100.0] vs. 80.8% [95% CI, 63.4–100.0], *P* = 0.197) (Figure 2D–F). (*All Figures, attached at the time of submission, were deleted due to space limitations. — BCT Editorial Office)*

Oral Presentation

Virtual Screening of Phytochemical Library of Indian Medicinal Plants for Transplant Associated Thrombotic Microangiopathy

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Aims: Transplant Associated Thrombotic Microangiopathy (TA-TMA) is characterized by extensive damage to the endothelium leading to endothelial dysfunction, microangiopathic hemolytic anemia, dysregulation and uninhibited activation of complement pathways leading to Membrane Attack Complex formation, finally leading to organ ischemia and subsequent failure. The incidence of TA-TMA ranges from 10-35% and the morality rate ranges from 40-90%. TA-TMA is the result of complex interplay of various prothrombotic and proinflammatory proteins. Phytochemicals offer a vast and diverse array of bioactive compounds with potential therapeutic effects. The aim of the study was to map out various proteins involved in the TA-TMA cascade, and identify phytochemicals obtained from indigenous plants that bind to these proteins through virtual screening (VS).

Methods: Proteins that are involved in the pathophysiology of TA-TMA were recognized through available literature and broadly accommodated under one of the four categories namely: proinflammatory, prothrombotic, oxidative stress induction and response, and Immunomodulatory proteins. Phytochemical library used for docking was chosen from commercially curated plant phytochemical libraries and Indian Medicinal Plants Phytochemistry and Therapeutics (IMPPAT) database 2.0. Approximately 2500 compounds were virtually screened for binding to target proteins through molecular docking. The proteins of particular interest were ICAM-1, VCAM, complement proteins such as C3 and C5 and thrombotic proteins such as Factor XA. Glide Emodel module of Schrodinger software was used for this process. Both Guided Docking and Site-mapping followed by docking were performed. In guided docking, sites and residues determined to be of structural and functional significance to a given protein were chosen for the creation of 'Receptor' sites. In site-mapping the software would identify ligand binding pockets on the target protein based on the volume and residual makeup of the binding pocket. The phytochemical compounds went through three stages of screening namely, High Throughput virtual Screening (HTVS), Standard Precision(SP) and Extra Precision

(XP). A cutoff docking score (binding energy) of -7.0 Kcal/mol or less was set to identify compounds with good affinity as per standard practice.

Results: Each target protein had at least one (Median = 13.5(1-25)) phytochemical ligand that docked with a binding energy of less than -7.0 Kcal/mol. Some compounds exhibited affinity for more than one target protein. Phytochemicals with binding energy below the cutoff were ranked and the top 20 compounds with least binding energies across all targets were picked for Molecular Dynamics Simulations (MDS) to study their binding characteristics in a dynamic system as opposed to static molecular binding.

Conclusions: TA-TMA is an orphan complication of hematopoietic cell transplant with few therapeutic options and high mortality. Phytochemicals as drug candidates are largely untapped. A number of phytochemicals found in indigenous Indian herbs were found to have good affinity for one or more protein targets implicated in the pathogenesis of TA-TMA. Further studies are ongoing using MDS to narrow down the leads for the next stage of screening using in vitro systems.

Funding: The study is funded by Indian Council of Medical Research.

Oral Presentation

Early Immunological Predictors of Cytomegalovirus (CMV) Infection After Allogeneic Hematopoietic Stem Cell Transplantation in a High Seropositive Population

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Aim:
Cytomegalovirus infection (CMV-I) is a common complication after allogeneic hematopoietic stem cell transplantation (HSCT), especially in CMV-seropositive populations. We set the study to determine CMV-I prevalence in this high seropositive population and evaluate the role of immune reconstitution (CD4 and CD8 T cells) and cell-mediated immunity on CMV-I using Quantiferon-CMV (QF-CMV).

Methods:
We conducted a prospective cohort study of 72 patients (aged 14–68) undergoing matched related donor HSCT for various haematological diseases between August 2022 and January 2024. Immunological assessments were performed on Days +15, +30, +60, +90, and +180. We used longitudinal mixed models and generalized equalizing equation to evaluate changes over time, and logistic regression to identify predictors of clinically significant CMV (CS-CMV), defined as CMV infection requiring pre-emptive treatment.

Results:
CS-CMV occurred in 65.3% of patients. Absence of QF-CMV reactivity at Day +30 was associated with increased CS-CMV risk (OR = 0.28, p = 0.023), while gastrointestinal tract involvement of acute GVHD was a strong predictor (OR = 5.57, p = 0.007). CD4 suppression was more pronounced in CS-CMV+ patients at Days 15 and 60. CD8 levels declined early in both groups but remained suppressed longer in CS-CMV– patients. Time point analysis showed significantly lower odds of CS-CMV+ at Day +60 (p = 0.042) and Day +90 (p = 0.016) compared to baseline (Day +180). QF-CMV status alone or in interaction with time did not significantly predict CS-CMV development, though a trend was observed at Day +90 (p = 0.091) in QF-CMV-negative individuals.

Conclusion:
QF-CMV non-reactivity at Day +30 and gastrointestinal GVHD are significant early

predictors of CMV infection. Time since transplant also independently influences risk, with lower odds of CS-CMV beyond Day +60. These findings support integrating immune markers and time-based risk into CMV monitoring strategies in high-seroprevalence populations.

Disclosures:

This research was supported by Global Investigator-Initiated Studies Program of Merck Sharp & Dohme Corp (MSD). The company had no role in analysing the data or preparing the abstract.

Oral Presentation

Haploidentical HSCT in Pediatric Leukemias: TCR Alpha/Beta Depleted Transplant vs. Post-Transplant Cyclophosphamide- A Single Center experience

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Haploidentical hematopoietic stem cell transplantation (haplo-HSCT) is an increasingly utilized transplant strategy for pediatric leukemias when matched related donors are not available. Two major approaches for graft-versus-host disease (GVHD) prophylaxis in haplo-HSCT include TCR alpha/beta depletion and post-transplant cyclophosphamide (PTCy). This study aims to compare the efficacy, safety, and outcomes of these two approaches in pediatric leukemia patients.

METHOD: This is a retrospective analysis of data of children with acute leukemias who underwent haplo-HSCT between January 2013 to December 2023 at our institution. Outcomes assessed included overall survival (OS), incidence of acute and chronic GVHD, relapse rates, and transplant-related mortality (TRM).

RESULTS: A total of 132 children with Leukemias underwent Haplo identical HSCT at our center in this study period. Table 1 depicts patient and transplant characteristics. Of 132, PTCy was used as GVHD prophylaxis in 74(56.1%) and TCR Alpha beta depletion in 58 (43.9%) children. Incidences of Acute GVHD were 50% and 41.37% in PTCy and TCR Alpha beta depletion group respectively . Median follow-up period was 325 days (Range 74-957.25 days). Relapse rates were 8.1% and 10.34% in PTCy and T cell-depleted transplants respectively. Overall TRM was 21.1% (21.6% in PTCy group and 20.6% in T cell-depleted group). Overall Survival for the entire cohort was 69.69% at a median follow-up of 325 days(70.27% in PTCy group and 68.96% in T cell depleted group). Overall event free survival was 68.93% at a median followup of 325 days (70.27% in PTCy group and 67.79% in T Cell-depleted transplant). Detailed results are depicted in Table 2. (*Table 2, attached at the time of submission, was deleted due to space limitations. —BCT Editorial Office*)

CONCLUSIONS: Haploidentical HSCT is an excellent alternative, when fully matched family donors are not available. Incidence of GVHD, Overall survival and relapse free

survival were comparable between PTCy group and TCR Alpha beta depletion groups.

Oral Presentation

Clinical Impact of Donor-Source on Allogeneic Hematopoietic Cell Transplantation for Childhood ALL: A Report from JSTCT Pediatric ALL-WG

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Background: The landscape of donor source selection for allogeneic hematopoietic cell transplantation (allo-HCT) is evolving due to demographic shifts such as declining birth rates and advancements in graft-versus-host disease (GVHD) prophylaxis. This study aims to update transplantation outcomes by donor source in pediatric ALL to aid clinical decision-making.

Methods: This study was conducted with approval from the centralized data committee and the Institutional Review Board of the National Center for Child Health and Development. We analyzed data from the TRUMP database for patients aged <16 years who underwent their first allo-HCT for ALL between 2013 and 2022 (n = 1,307). Patients were categorized into two cohorts: those undergoing transplant in first or second complete remission (standard-risk group, SR; n = 752) and those receiving transplant in non-remission or beyond second remission (high-risk group, HR; n = 555). Donor sources included HLA-matched sibling donors (MSD), HLA 7-8/8 matched related donors (7-8MRD), unrelated donors (UR), unrelated cord blood (UCB), post-transplant cyclophosphamide (PTCY)-haploidentical donors (HAPLO), and other haploidentical donors (other-HAPLO). Disease-free survival (DFS), overall survival (OS), and GVHD-free relapse-free survival (GRFS; defined as the absence of grade 3-4 acute GVHD, extensive chronic GVHD, relapse, or death) were analyzed using the log-rank test with MSD as the reference.

Results: The median follow-up period for survivors was 4.3 years. In the SR cohort, 3-year DFS/OS rates were: MSD (n = 79) 73%/87%; 7-8MRD (n = 66) 71%/79%; UR (n = 216) 78%/85%; UCB (n = 304) 72%/81%; and PTCY-HAPLO (n = 27) 71%/82%, all showing no significant difference from MSD. However, other-HAPLO (n = 60) had significantly lower DFS (55%, P = 0.02) and OS (63%, P = 0.007). The 3-year GRFS rates were: MSD 73%; 7-8MRD 48% (P = 0.004); UR 60% (P = 0.016); UCB 65% (P = 0.14); PTCY-HAPLO 59% (P = 0.18); and other-HAPLO 46% (P < 0.001), with UCB and PTCY-HAPLO showing comparable GRFS to MSD. In the HR cohort, 3-year DFS/OS/GRFS rates were: MSD (n = 31) 45%/47%/44%; 7-8MRD (n

= 58) 49%/54%/33%; UR (n = 89) 57%/66%/45%; UCB (n = 171) 41%/48%/37%; PTCY-HAPLO (n = 22) 32%/43%/26%; and other-HAPLO (n = 184) 29%/32%/15% ($P < 0.001$), with no significant differences except for the markedly lower GRFS in other-HAPLO.

Conclusion: This study suggests that for pediatric ALL undergoing first allo-HCT in remission, 7-8MRD, UR, UCB, and PTCY-HAPLO serve as viable alternatives to MSD in terms of DFS and OS. However, GVHD control remains a major challenge in non-MSD transplants.

POSTER PRESENTATION

Poster Presentation

Acute Leukemia

Outcomes and treatment determinants in intermediate-adverse risk acute leukaemia: Analysis of barriers to allogeneic stem cell transplant

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Aim: To evaluate outcomes among patients with intermediate- or adverse-risk acute myeloid leukaemia (AML) or acute lymphoblastic leukaemia (ALL) treated with intensive chemotherapy in the Northern region of New Zealand. This study also aims to identify and analyse relevant factors that determine whether patients proceed to allogeneic stem cell transplantation (alloSCT).

Method: We conducted a retrospective cohort study of adults diagnosed with intermediate- or adverse-risk AML or ALL from 2018 to 2023 in the Northern Region of New Zealand. Eligible cases were identified from the regional Leukaemia Multidisciplinary Meeting (MDM). Demographic data were analysed to determine if there were factors limiting access to alloSCT. Analyses included univariate analysis and binary logistic multivariate analysis for determinants of access to alloSCT as well as overall survival (OS) and progression-free survival (PFS).

Results: A total of 121 patients (AML = 86, ALL = 35) were included in the analysis. Median follow-up estimated using the reverse Kaplan-Meier method was 47.4 months (95% CI: 37.2–57.6) for patients who underwent alloSCT and 68.0 months (95% CI: 54.6–81.3) for those who did not. Median OS was not reached in the alloSCT group, compared to 8.6 months (95% CI: 5.8-11.4) in the non-SCT group. The 1-year OS was significantly higher for alloSCT recipients at 87.5% (95% CI: 79.9–95.1) versus 42.9% (95% CI: 29.1–56.7) for non-SCT ($p < 0.0001$). Similarly, 1-year PFS was 77.8% (95% CI: 68.0–87.6) for the alloSCT group and 28.6% (95% CI: 15.7–41.5) for the non-SCT group ($p < 0.0001$).
In multivariate analysis, younger age at diagnosis (OR 0.94, 95% CI: 0.89–0.99, p=0.019), a greater number of chemotherapy cycles prior to transplant (OR 2.01, 95% CI: 1.14–3.55, p=0.016) and shorter time from diagnosis to multidisciplinary meeting (OR 0.50, 95% CI: 0.26–0.97, p=0.040) were independently associated with increased odds of receiving an alloSCT. Māori and Pacific Peoples had significantly lower odds of receiving alloSCT (0.21, 95% CI: 0.06–0.77, p=0.018). Rurality or

distance from domicile to transplant hospital was not associated with access to alloSCT.
The primary reasons for not receiving alloSCT were disease progression (36.7%), lack of fitness (24.5%), and, equally, lack of donor, infection, and patient choice (12.2% each). One patient had treatment intensified and did not require transplantation.
Following the implementation of a "first-referred, first-transplanted" prioritisation waitlist in July 2021, the proportion of patients receiving alloSCT increased from 51.5% to 69.1% (p=0.05). However, the median time from diagnosis to alloSCT was longer after implementation, at 6.0 months (3.6–14.5) compared to 4.7 months (3.4–13.7), likely reflecting increased demand for transplantation services.

Conclusion: In the context of New Zealand's growing demand for alloSCT, this study demonstrates that transplantation remains critical for patients with higher risk acute leukaemia. Despite increasing referral volumes and resource pressures, access to alloSCT has improved with recent changes to prioritisation processes. However, delays in time to transplant reflect the challenge of meeting higher demand. Ultimately, alloSCT improves survival for high-risk patients, highlighting the need for continued investment and system adaptation to ensure equitable, timely access.

Poster Presentation

Acute Leukemia

Characteristics and Treatment Outcome of Extramedullary Relapse Involving Central Nervous System after Allogeneic Hematopoietic Cell Transplantation in Adult Patients with Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia

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Aims: The central nervous system (CNS) is the most important and unique site of extramedullary disease in adults with acute lymphoblastic leukemia (ALL). Our recent report demonstrated that 5 or 6 times of standard intrathecal therapy and allogeneic hematopoietic cell transplantation (allo-HCT) could prevent CNS relapses (CNSR) with the incidence of less than 5%, but most of them (~80%) occurred in Philadelphia chromosome (Ph)-positive ALL.

Methods: Among the 327 transplants of Ph-positive ALL from 2011 to 2022, we found 31 (9.4%) patients (median age 41 years, range 20-60) who developed CNSR after allo-HCT. CNSR was detected by MRI findings and CSF study, and bone marrow biopsy was also conducted. Treatment strategy for CNSR for Ph-positive ALL consisted of at least one time of intrathecal therapy (IT) followed by radiotherapy (RT, 24Gy/10Fx for brain, and 20Gy/10Fx for spine). According to the previous tyrosine kinase inhibitor (TKI) lines before CNSR, CNS-penetrating dasatinib (n=20) or ponatinib (n=7) were administered and other chemotherapy (MEC 1, Inotuzumab 2, or blinatumomab 1) was selected if all approved TKIs were used.

Results: Among the 31 Ph-positive ALL with CNSR after allo-HCT, 12 (38.7%) were major transcript *BCR/ABL1*, 27 (87.1%) were with additional chromosomal abnormalities, 15 (48.4%) were with extramedullary disease (CNS in 1) at diagnosis, and all were in CR before allo-HCT (10 [32.3%] CMR, 6 [19.4%] MMR, and 15 [48.4%] PMR). MAC regimen was used in 18 (58.1%) and acute and chronic GVHD was preceding in 12 (38.7%) and 13 (41.9%), respectively. As of relapse pattern, 19 were isolated CNSR (17 with MRD-positive, 2 with MRD-negative), while 12 were accompanied with or following bone marrow relapse (BMR). Isolated CNSR well responded to TKI-based local therapy in 89.5%, while CNSR+BMR responded in 58.3% and early death occurred in 33.3%. Dasatinib-base therapy showed CR in

75% (MRD response in 92.8%), ponatinib-based therapy showed CR in 100% (MRD response in 85.7%), and others showed CR in 50%. Median F/U duration of 24 months, 2-year OS was 37.9%, and for isolated CNSR, it was 48.1%. Pre-HCT PMR was significantly related with concomitant BMR and poor survival outcome. Second allo-HCT was conducted in 4 patients, but all died due to leukemic progression and transplant complications.

Conclusions: Our data showed Ph-positive ALL was significantly related with CNSR after allo-HCT and TKI-based chemo-free local therapy using IT plus RT demonstrated feasible treatment outcome especially in isolated CNSR.

Poster Presentation

Acute Leukemia

Time from Diagnosis to Treatment as a Prognostic Factor in Adult Patient Newly-Diagnosed with Acute Myeloid Leukemia: A 5-Year Retrospective Study in a Tertiary Institution

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BACKGROUND: Acute Myeloid Leukemia (AML) is a rapidly progressing hematologic malignancy requiring timely intervention. While international studies have produced conflicting evidence whether time from diagnosis to treatment (TDT) impact outcomes, this study investigates the prognostic effect of TDT in a resource-limited setting.

METHODS: This retrospective cohort study reviewed adult AML patients (≥19 years) diagnosed and treated with intensive chemotherapy in a government tertiary hospital between 2020 and 2024. Clinical, demographic, and treatment data were collected and analyzed. Patients were grouped by TDT intervals: 0-30, 31-60, and >60 days. Key outcomes included complete remission (CR/CRi), early death (ED), event-free survival (EFS), and overall survival (OS). Multivariate regression and Cox proportional hazards models were used to assess associations.

RESULTS: Among 72 patients (median age 37 years), median TDT was 26.5 days. CR/CRi was achieved in 51.4%, with no significant differences across TDT groups. ED occurred in 47.2% overall, increasing with longer TDT. Median follow-up was 5.7 months; two-year OS was 4.14%. Multivariable analysis revealed no significant association between TDT and CR/CRi, ED, EFS, or OS. Common causes of delay included infection and cost barriers.

CONCLUSION: In this cohort, TDT was not independently associated with clinical outcomes in AML, aligning with prior international studies and meta-analysis suggesting that while prompt treatment is ideal, delays in initiation may not independently predict prognosis, especially in resource-limited settings where infections and systemic barriers contribute to treatment delay. High early mortality emphasizes the need to address systemic barriers to timely care.

Poster Presentation

Acute Leukemia

Case Report: Autologous Hematopoietic Stem Cell Transplantation in Blastic Plasmacytoid Dendritic Cell Neoplasm The First Case Diagnosed in Vietnam

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Background:
Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) is a rare hematological tumor originating from plasmacytoid cells. It is an aggressive disease with a short median survival, so early detection and treatment are crucial. Currently, CD123 inhibitors have recently been approved for first-line treatment, and consolidation with allogeneic hematopoietic stem cell transplantation (HSCT) can provide a curable therapy. However, access to the drug remains challenging, and not all patients are suitable for allogeneic hematopoietic stem cell transplantation (HSCT). This article describes the first case of BPDCN diagnosed in Vietnam and consolidated with autologous hematopoietic stem cell transplantation after response with traditional chemotherapy.

Case presentation:
We report that a 69-year-old man had felt itchy one month before hospital administration. Subsequently, multiple skin lesions of varying sizes and shapes, ranging from small bruise-like papules to purple nodules, were seen on his arms and shoulders. A skin biopsy of the lesion revealed atypical medium-sized blastoid tumor cells with indistinct nuclei infiltrating the dermis and subcutaneous fat. The tumor cells were diffusely positive for CD45, CD4, and CD56 but negative for CD3, CD20, and CD79a in immunohistochemistry. MPO and lysozyme were totally negative in the tumor cells, but CD123, TCL1, and TCF4 were strongly positive. The diagnosis of BPDCN was established according to the 5th edition of the WHO classification of hematolymphoid tumors. Due to his advanced age, he was treated with six cycles of CHOP chemotherapy and achieved a complete response at the end of the treatment. At this point, the inability to perform an allogeneic stem cell transplant and observation without further treatment could result in a rapid relapse. Therefore, we decided to perform an autologous stem cell transplant as a consolidation therapy to prolong the remission stage. We mobilized hematopoietic stem cells by combination chemotherapy and G-CSF. The collection of CD34

hematoietic stem cells was 5.6 x 10^6/kg after two peripheral blood leukapheresis procedures. This patient underwent the BeEAM conditioning regimen. During the transplantation, the patient experienced thrombocytopenia and severe neutropenia on days +1 and +2, respectively. On days +9 and +10, respectively, the neutrophils and platelets recovered. Adverse effects were neutropenic fever and severe oral mucosal ulcers. After HSCT, he is still alive and without any signs of relapse at the 6-month follow-up.

Conclusion:

BPDCN is a challenging disease to diagnose and treat due to limited resources, especially in Vietnam.Autologous stem cell transplantation is a potential option for consolidation in elderly BPDCN patients who respond to induction therapy but are not suitable for allogeneic stem cell transplantation.

Poster Presentation

Acute Leukemia

Transplant Outcomes After HLA Class I Allele-Mismatched Unrelated Versus Haploidentical Donors in Acute Myeloid Leukemia

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Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is a curative option for acute myeloid leukemia (AML), but alternative donors such as 1 allele-mismatched unrelated donors (1-MMUD) and haploidentical donors (HID) are considered when matched donors are unavailable. While both donor types have shown comparable outcomes to HLA-matched unrelated donors, direct comparisons between 1-MMUD and HID remain limited.

We retrospectively analyzed AML patients who underwent first allo-HSCT from either a 1-MMUD (n=108) or HID (n=447) between 2002 and 2022 at a single institution. All patients were in complete remission (CR or CRi) at transplant and received T-cell replete peripheral blood stem cells. Conditioning regimens for HID included fludarabine, busulfan, and total body irradiation (TBI), with GVHD prophylaxis comprising antithymocyte globulin, methotrexate, and calcineurin inhibitors. Conditioning for 1-MMUD varied between myeloablative and reduced-intensity approaches.

Baseline characteristics were generally similar, although the 1-MMUD group was younger (median 42 vs. 51 years, $p < 0.01$) and had lower ABO matching (28.7% vs. 51.7%, $p < 0.01$). Median overall survival (OS) was comparable (60.1 months for 1-MMUD vs. 58.7 months for HID, $p = 0.81$), as was relapse-free survival (RFS; 54.8 vs. 40.9 months, $p = 0.96$). The 60-month cumulative incidence of relapse (26.1% vs. 25.7%) and non-relapse mortality (23.9% vs. 26.3%) did not differ significantly. Acute GVHD (grade ≥2) rates were similar (38.0% vs. 45.4%, $p = 0.41$), but chronic GVHD incidence was significantly higher in the HID group (63.6% vs. 52.0%, $p < 0.01$). In multivariate analysis adjusting for age, cytogenetics, donor sex mismatch, and ABO incompatibility, there was no significant difference in OS between the two donor types (HR for 1-MMUD vs. HID: 1.25, 95% CI: 0.92–1.69, $p = 0.15$).

1-MMUD and HID provide comparable outcomes in AML patients undergoing allo-HSCT, though HID may be associated with higher chronic GVHD risk. These findings

support the use of either donor type as viable alternatives in the absence of fully matched donors. Further prospective studies, including use of post-transplant cyclophosphamide, are needed to refine donor selection strategies.

Poster Presentation

Acute Leukemia

Feasibility of Non-Cryopreserved Autologous Stem Cell Transplant for Acute Myeloid Leukemia: Experience from Myanmar

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BACKGROUND: Acute myeloid leukemia (AML) is the most common form of acute leukemia among adults and allogenic stem cell transplant (Allo-HSCT) is the only curative treatment for high-risk AML. In patients who are ineligible for Allo-HSCT, autologous stem cell transplant (ASCT) is one of the established treatments. Although cryopreservation is part of a standard procedure for ASCT, it has shortcomings like high cost, infusion-related toxicities and affecting cell viability and engraftment. Under current resource poor situation in Myanmar, this study was performed to evaluate the feasibility of non-cryopreserved ASCT for AML patients.

METHODS: The AML patients with undetectable minimal residual disease (MRD) after induction therapy, who could not proceed for Allo-HSCT for various reasons, were offered for autologous stem cell transplant. After peripheral blood stem cell mobilization, the collected stem cell bags were stored without cryopreservation at two to four degree celsius in a blood bank refrigerator. Busulfan based conditioning protocols, modified to complete within four to five days, were given. The stored stem cells were reinfused after conditioning. The data of these patients, who underwent ASCT between January 2024 and December 2024, were retrospectively reviewed.

RESULTS: A total of six patients, three males and three females, received peripheral blood ASCT without cryopreservation. The median age was 29 years (range 14 – 41 years). One third of the cases was relapsed AML. There were no comorbidities. The conditioning protocols were Busulfan-Etoposide-Cytarabine (BEA) in five cases and Busulfan-Melphalan (Bu-Mel) in one case. The median stem cell dose, calculated on viable CD34 positive cells, was 4.4 x 10^6 cells/kg (range 2.7 – 9.1 x 10^6 cells /kg). The median time interval between harvest to reinfusion was 119.75 hours (range 82 – 148 hours). The median days for neutrophil and platelet engraftment were day+23 (range 13 – 25 days) and day+22 (range 15 – 41 days) respectively. There were no post-transplant complications. Except one case who lost follow-up after day+65, the remaining five cases achieved undetectable MRD at day+100. The median follow-up

period was eight months (range seven – 16 months). One case relapsed at six months post-transplant. The remaining four cases (66.67%) were in remission at the end of this study period.

CONCLUSION: Based on these observations, ASCT without cryopreservation was feasible for AML cases and achieved engraftment in reasonable time with relapse free survival in majority of the patients. More studies are needed to confirm safety and efficacy so that it can be applied as a practice of ASCT for AML.

Poster Presentation

Acute Leukemia

Bridging Survival Gaps: The Impact of Allogeneic Transplantation in Intermediate- and Adverse-Risk Leukaemia in Northern New Zealand

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Aim: To assess transplant outcomes in patients with intermediate- or adverse-risk acute myeloid leukaemia (AML) or acute lymphoblastic leukaemia (ALL) who underwent allogeneic stem cell transplantation (alloSCT) following intensive chemotherapy in the Northern region, New Zealand. Demand for alloSCT has increased in New Zealand with challenges to deliver this service. This study aims to provide insight into real-world outcomes and the evolving landscape of alloSCT delivery in Aotearoa New Zealand with a focus on patients with intermediate and adverse risk leukaemia.

Method: The prevalence of AML subtypes was reported, and survival outcomes were compared by disease type. Transplant characteristics, overall survival (OS), progression-free survival (PFS), and the cumulative incidence of transplant-related mortality (TRM), relapse, acute graft-versus-host disease (aGVHD), and chronic graft-versus-host disease (cGVHD) were analysed. Competing risks methodology was used for cumulative incidence analyses, with death and relapse considered as competing events where appropriate.

Results: Among 72 patients who underwent allogeneic stem cell transplantation, 88.9% were transplanted in first complete remission and 11.1% in second remission. Most donors were unrelated (44.4%), peripheral blood was the graft source in 98.6% of cases, and myeloablative conditioning was used in 61.1%. Neutrophil and platelet engraftment was achieved 95.8% and 90.3%, respectively, with a median engraftment time of 19 days for both. Within the AML cohort, 16.7% had transformed from MDS/MPN and 6.3% were TP53 mutated. The most common AML subtype was myelodysplasia-related (43.8%), followed by NPM1-mutated (14.6%), post-cytotoxic therapy (12.5%), and KMT2A-rearranged (8.3%). According to ELN 2017 criteria, 66.7% were classified as adverse risk and 33.3% as intermediate risk.

At 1-year, overall survival was 87.5% (95% CI: 80.0–95.1), progression-free survival was 77.8% (68.2–87.4), treatment-related mortality was 9.7% (4.2–17.9), and

relapse incidence was 18.2% (10.2–27.9). The leading cause of death was disease recurrence or progression (23.6%), followed by infection (5.6%), GVHD (2.8%), and organ or graft failure (4.2%). Ongoing immunosuppression was required in 25.4% of patients alive at 12 months.

Conclusion: Allogeneic stem cell transplantation in Northern New Zealand achieves favourable 1-year survival in this cohort of higher risk acute leukaemia. Relapse remains the leading cause of mortality, emphasising the importance of improving access to post-transplant care and relapse prevention strategies across Aotearoa.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Autologous Stem Cell Transplant in Non-HEPA Filtered, Non-specialized Single-bed Wards, a Case Report

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International guidelines suggest that patients who undergo stem cell transplant must be admitted in specialized isolation units to limit morbidities associated with the procedure, such as infection. These facilities are however, very limited and remain generally inaccessible in developing countries, leading to delayed transplantation. To address the lack of facilities, cases of stem cell transplant in non-specialized rooms have been attempted, although there is still sparse local literature documenting such cases. This case report discusses a patient with Hodgkin's lymphoma who underwent transplantation in a non-HEPA filter, non positive pressure environment. Studies on similar cases have had various results, many of which show the its feasibility. The case above provides insight on stem cell transplant and its success in regular rooms. It is deemed feasible and such procedure may be reproducible in many institutions in developing countries where specialized facilities are limited.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Case Series Report: Autologous Hematopoietic Cell Transplantation In Mantle Cell Lymphoma At Blood Transfusion Hematology Hospital in 10 Years

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Background:
Mantle cell lymphoma is an uncommon subtype of non-Hodgkin lymphoma. Clinical presentations and outcomes are heterogeneous. The optimal treatment strategy for young and fit patients consists of three phases: induction, consolidation, and maintenance. For patients who respond to first-induction chemotherapy, consolidation with autologous hematopoietic stem cell transplantation (AHCT) plays a crucial role in prolonging remission and controlling disease.

Aims:
Initial evaluation of the effectiveness of autologous hematopoietic stem cell transplantation in patients with mantle cell lymphoma.

Method:
A retrospective case series report of mantle cell lymphoma consolidated with autologous hematopoietic stem cell transplantation at Blood Transfusion Hematology Hospital for 10 years from 2015-2025.

Results:
Over a ten-year period, the Blood Transfusion Hematology Hospital diagnosed and treated 51 patients with mantle cell lymphoma. Only ten of these patients were consolidated with autologous stem cell transplantation following 4–8 cycles of multiagent chemotherapy that included rituximab. The ECOG scores of these ten patients ranged from 0 to 1, and their ages ranged from 41 to 60. Nine out of ten patients had a complete metabolic response prior to transplantation. 8 patients use only G-CSF to mobilize hematopoietic stem cells from bone marrow to peripheral blood. The average dosage of CD34-positive stem cells administered is 6.2 x 106/kg. The conditioning regimen BeEAM was used in 8/10 patients, and the remainder used BuCyE (2/10). The main side effects that occurred during transplantation were severe oral mucosal ulcers, neutropenic fever, and infection. The infectious agents

recorded were E. coli (1/10), Candida albicans (2/10), and Cytomegalovirus (1/10). All patients experienced engraftment with a complete response according to PET-based assessments per Lugano criteria and were kept on maintenance with rituximab monotherapy. Up to now, the median follow-up time is 50 months; there are 2 patients who relapsed 1 year after transplantation and died thereafter. 8/10 patients are still alive without any sign of relapse, with remission duration ranging from 11 to 113 months. Of 8 patients in the complete remission stage, two patients have had remission lasting more than nine years.

Conclusions:
AHCT consolidation is an effective and tolerable treatment for mantle cell lymphoma.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Real-World Outcomes of Diffuse Large B-Cell Lymphoma Patient With or Without Myc Gene Rearrangement Treated With R-CHOP Regimen at Ho Chi Minh City Oncology Hospital

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Aims: To research the influence of single MYC and double MYC and BCL2/BCL6 gene rearrangements on treatment outcomes of diffuse large B-cell lymphoma (DLBCL) patients treated with R-CHOP regimen.

Methods: We retrospectively studied 39 patients who were diagnosed with DLBCL equal to or over 16 years old and treated with the R-CHOP regimen at the Department of Medical Oncology 1, Ho Chi Minh City Oncology Hospital from September 1, 2017 to March 31, 2018. All patients were tested with FISH to find MYC, BCL2, BCL6 gene rearrangements and were divided into three groups: MYC+ (group 1), MYC+ and BCL2/BCL6+ (group 2) and MYC- (group 3).

Results: There were 23 male and 16 female patients, male:female ratio was 1.43:1. Median age was 55,3 years (17,5-73,1 years). The presence of B symptoms accounted for 41%. Neck lymph node (92,5%) was the most common site of lymph node involvement, while tumor of gastrointestinal tract (30,4%) was common for extranodal involvement. Most patients belong to non-germinal centre B-cell like subgroup by Han's criteria (53,8%). The MYC gene rearrangements rates of group 1, 2 and 3 were 10,4% (5/39 cases), 4,2% (2/39 cases) và 66,7% (32/39 cases), respectively. The rate of elevated LDH was 43,6%, of stage III-IV 41% and of patients with intermediate-high and high risk was 23,1%. Chemotherapy alone accounted for 37 cases (94,9%); chemotherapy combined radiotherapy accounted for 2 cases (5,1%). The complete response rate was 76.9%. The rate of neutropenia, grad 3-4 was 28,2%. The 3-year progressive-free survival (PFS) rate was 68.9% and the 3-year overall survival (OS) rate was 76,2%. The patients with MYC gene rearrangement significantly reduced the 3-year OS rate: group 1 (MYC+) 50%, group 2 (MYC and BCL2/BCL6+) 0% when compared with group 3 (MYC-) 84,4% (p = 0.007), however that did not affect PFS (p = 0,09). Multivariate analysis showed that

three factors: presence of B symptoms (p = 0,028), elevated LDH (p = 0,042) and MYC gene rearrangement (p = 0,005) were independent predictors of OS.

Conclusion: We recommended that in DLBCL patients with single MYC or MYC and BCL2/BCL6 gene rearrangements (double hit lymphoma) treated with R-CHOP regimen, HDT and ASCT or intensification chemotherapy should be considered to increase OS for these patients.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Application of Comprehensive Nursing Management in CAR-T Therapy for Patients with Relapsed/Refractory Lymphoma

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Objective:
To apply a comprehensive nursing management model in CAR-T therapy for patients with relapsed/refractory lymphoma, providing precise nursing care and supporting patients throughout the treatment process.

Methods:
A total of 505 patients were included, comprising 271 males and 234 females, with a median age of 51 years (range: 13–86 years). Diagnoses included: diffuse large B-cell lymphoma (DLBCL) in 342 cases, high-grade B-cell lymphoma (HGBL) in 29 cases, primary central nervous system lymphoma (PCNSL) in 33 cases, primary mediastinal B-cell lymphoma (PMBL) in 19 cases, Burkitt lymphoma (BL) in 20 cases, and other types in 62 cases. Ann Arbor stage ≥ III was observed in 459 cases (90.8%), and ECOG performance status ≥ 2 in 173 cases (34.2%). A total of 369 patients (72.8%) had an International Prognostic Index (IPI) score ≥ 2. From day -7 to -5, lymphodepletion chemotherapy was administered. On day 0, CAR-T cells were infused with a dual verification process by medical and nursing staff. The median CAR-T cell dose was 1.57 × 10^6/kg (range: 0.00133–98 × 10^6/kg).

Comprehensive nursing management during CAR-T therapy included:

- Pre-, intra-, and post-apheresis care for peripheral blood lymphocyte collection.
- Nursing care during lymphodepletion, including medication management, lab monitoring, infection prevention, and basic nursing care.
- Nursing care during CAR-T cell infusion, involving nursing assessments, CRS grading and management, ICANS grading and management, care for tumor-related edema, and infection prevention.
- Ongoing support including nutritional guidance, activity recommendations, health education, and psychological support.

- Post-treatment care included discharge education, discharge planning, and follow-up management.

Results:
After CAR-T therapy, 388 patients (76.6%) developed cytokine release syndrome (CRS), with 41 cases (8.1%) classified as grade ≥3. ICANS occurred in 22 patients (4.3%), including 13 cases (2.5%) of grade ≥3. Grade ≥4 neutropenia was observed in 431 patients (85.3%), grade ≥4 thrombocytopenia in 243 patients (48.1%), and grade ≥3 anemia in 299 patients (59.2%). Therapeutic evaluation post-treatment showed that 312 patients (61.7%) achieved tumor remission. The median follow-up period was 381 days (range: 4–1959 days), and the median overall survival was 33.44 months. The 3-year and 5-year overall survival (OS) rates were 49.0% and 47.3%, respectively.

Conclusion:
In patients with relapsed/refractory lymphoma undergoing CAR-T therapy, common adverse events include grade 1–2 CRS, pancytopenia, and infection risk. Nurses who are proficient in comprehensive nursing management play a vital role in monitoring disease changes, lab indicators, and providing consistent health care guidance and support throughout all treatment stages and in managing treatment-related toxicities.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

A Case Report on Complete Healing of a Tumor Wound in a Patient with Refractory/Relapsed Peripheral T-cell Lymphoma

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Objective:
To share the nursing experience of complete tumor wound healing in a patient with refractory/relapsed peripheral T-cell lymphoma (PTCL).

Methods:
The patient was a 54-year-old female diagnosed with peripheral T-cell lymphoma, Ann Arbor stage IVA. She was admitted on October 9, 2024. Physical examination revealed diffuse erythema across the body, more prominent above the waist, accompanied by pruritus. A tumor wound on the left hip measured approximately 5×3 cm, purple-red in color, with surrounding erythema and swelling, and a small amount of exudate. Pain was rated 1 on the NRS scale, ECOG performance status was 1, and NRS 2002 score was 2. The chemotherapy regimen consisted of liposomal mitoxantrone + methotrexate (MTX) + dexamethasone combined with targeted therapy. The patient experienced intermittent fever during treatment, with a peak temperature of 38.8°C. Infection was ruled out, and the fever was considered tumor-related.
Wound care was conducted in three phases:

Tumor progression phase: The wound exhibited redness and swelling around the area, slight exudate, and stinging pain. The primary goal during this phase was infection prevention. Daily dressing changes were performed using saline for cleansing, followed by povidone-iodine disinfection, saline wipe-down, application of silver sulfadiazine dressing, and gauze dressing for coverage.
Tumor control phase: The periwound area turned from red and swollen to dark brown, with no exudate or pain, and the wound bed showed yellowish tissue. The main focus was debridement. Dressings were changed twice weekly. Kangfuxin solution was applied as a wet compress for 15 minutes. After drying, debridement gel and zinc sulfadiazine were applied at a thickness of approximately 0.5 cm, followed by silver sulfadiazine dressing and gauze coverage.

Wound healing phase: The wound bed showed red granulation tissue. The focus was to promote granulation and epithelial tissue growth. Dressing changes continued twice weekly. Kangfuxin solution wet compresses were applied for 15 minutes, followed by zinc sulfadiazine ointment (0.5 cm thick), and gauze dressing.

Throughout the treatment, laboratory values were monitored to prevent infection and bleeding. Body temperature was tracked for symptomatic antipyresis. Nutritional guidance, activity instruction, and psychological support were also provided.

Results:

Following chemotherapy, the patient developed hematologic toxicities: grade 1 hemoglobin reduction, grade 4 neutropenia, and grade 3 thrombocytopenia. After two cycles, PET/CT indicated a partial response (PR) of the primary disease. Following four cycles, the diffuse erythema had faded and turned into residual dark brown lesions. The tumor wound had completely healed.

Conclusion:

In patients with PTCL undergoing chemotherapy, tumor wounds are prone to ulceration with a high risk of infection and present significant nursing challenges. A close collaboration between comprehensive treatment and precision nursing is essential. Stage-specific sterile dressing protocols combined with debridement gels, antibiotic ointments, and advanced wound dressings can effectively promote wound healing.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Autologous Stem Cell Transplantation for High-Risk Young Adult Patient with Systemic Anaplastic Large Cell Lymphoma

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Introduction: CD30 expression and other common pathologic characteristics define the diverse group of very uncommon T-cell non-Hodgkin lymphomas known as systemic Anaplastic Large Cell Lymphomas (sALCLs). It is a rare variety with an aggressive feature. Based on the expression of the Anaplastic Lymphoma Kinase (ALK) protein, sALCLs can be divided into two main groups: ALK-negative and ALK + positive. ALK-negative ALCL are typically middle-aged patients, and their 5-year overall survival rate is less than 50%. Here, we present the case of young adult sALCLs who underwent Autologous Stem Cell Transplantation (ASCT) at Dharmais National Cancer Center Hospital Indonesia.

Case Presentation: A 27-year-old woman was diagnosed with sALCLs with ALK-negative in 2024. He received the R-CHOP (Rituximab, Cyclophosphamide, Doxorubicin, Vincristine, and Prednisone) chemotherapy for six cycles, achieving complete remission (CR). In March 2025, the patient underwent conditioning chemotherapy with BEAM (Carmustine, Cytarabine, Etoposide, and Melphalan) regimen, followed by ASCT. A total of 8.78 x 106 /kg bodyweight CD34+ PBSC was administered on Day 0. Neutropenic fever happened on Day 8 but then subsided after the administration of antibiotics. Engraftment was achieved on Day 13 and the patient was sent home the day after.

Discussion: ASCT is a potential treatment option for sALCLs ALK-negative patients who achieved their first remission after induction chemotherapy. It's often considered a consolidation therapy to reduce the risk of relapse. In existing guidelines, ASCT is recommended in relapsed/refractory sALCLs. While the role of ASCT in the upfront setting is still debated, ASCT may be considered in patients who have high-risk features. Because this patient has a high risk as indicated by the presence of negative ALK, the MDT team decided to perform ASCT. Studies have shown that ASCT in first remission can lead to improved progression-free survival (PFS) and overall survival (OS) in ALCL patients, particularly those with high-risk

features. The patient's disease-free period will be measured and reported periodically.

Conclusion: ASCT may provide a benefit in specific clinical scenarios such as patients with sALCs ALK-negative cases. It is possible for Indonesia to implement a high-quality and safe ASCT. The largest obstacle to overcome is the financial component.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Outcomes of High-Dose Chemotherapy and Autologous Stem Cell Transplantation (ASCT) in Relapsed/Refractory (RR) Diffuse Large B cell lymphoma (DLBCL): A 22-Year Single-Centre Experience from Singapore

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Background/ Aims:
ASCT has historically been standard of care for RR DLBCL. Recent advances in the field such as bispecific therapy and chimeric antigen receptor T cell therapy (CAR T), have challenged the continued role of ASCT in the treatment algorithm for RR DLBCL. We thus examine the real world outcomes of ASCT in our multi-ethnic Asian population, so as to assess the real-world clinical need for new therapeutics in this disease entity, and to establish a benchmark upon which future therapeutics can be compared against.

Methods:
We conducted a retrospective analysis involving patients (N=127) with relapsed/refractory DLBCL who underwent ASCT as consolidation after salvage therapy, at the National University Cancer Institute Singapore, between 2002 and 2024. Clinical outcomes including overall survival (OS), progression-free survival (PFS), and relapse rates were evaluated using Kaplan–Meier and cumulative incidence analyses. Stratified survival analyses were performed based on pre-transplant disease status and timing of relapse.

Results:
The cohort was predominantly male (59%) with a median age at transplant of 56 years (range: 17–82); 17% of patients (N=22) were older than 65 years. 7% of patients (N=9) had CNS involvement and received a BCNU-thiotepa conditioning, while all other patients received BEAM conditioning. 73% (N=94) were transplanted in CR. With a median follow up of 4.2 years, the median OS was 14.4 years, with a 5-year OS rate of 68.4% (95% CI: 59.4–75.9), while the median PFS was 11.9 years, and the 5-year PFS rate was 61.3% (95% CI: 52.1–69.2). Cumulative

incidence of relapse was 24.6% at 1 year and 31.1% at 2 years. Non-relapse mortality (NRM) was low at 4.8% at 1 year and 6.4% at 2 years. Patients who achieved a complete remission status (CR) prior to ASCT had significantly better outcomes compared to those in partial remission (PR) with a higher median OS and PFS (median OS 15.5 years versus 5.7 years; p= 0.03, and median PFS 12.7 years vs 1.7 years, p=0.08 respectively). Multivariate analysis identified remission status at ASCT and age at ASCT as independent prognostic factors for OS while age was an independent prognostic factor for PFS. In contrast, neither year of transplant nor timing of relapse of DLBCL (< or >12 months from initial diagnosis) had impact on PFS or OS. Notably, amongst the subgroup of patients with early relapses (≤12 months from diagnosis) who achieved CR or PR with salvage, the 5-yr PFS and OS rates remained encouraging, at 60% and 66% (for patients in CR, N=27) and 48% and 57% (for patients in PR, N=33) respectively. For patients with CNS involvement, the 5-yr PFS and OS was 33% and 33% respectively.

Conclusion:

In conclusion, this real world study supports the long-term efficacy and safety of ASCT in patients with relapsed/refractory DLBCL, including those with early relapses (<12 months) as well as those CNS involvement. These findings reaffirm ASCT as a viable and potentially curative approach in the modern era, though patient selection remains critical.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Evaluating the Role of Autologous Stem Cell Transplantation in Lymphoma: A Malaysian Centre's Experience

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Introduction: High-dose chemotherapy followed by autologous stem cell transplantation (ASCT) is an established therapeutic strategy to improve overall survival (OS) in patients with relapsed lymphoma. However, the procedure is associated with a non-negligible risk of treatment-related mortality (TRM), highlighting the need for stringent patient selection to maximize therapeutic benefit.

Aim: To evaluate the clinical outcomes and therapeutic role of autologous stem cell transplantation (ASCT) in the management of patients with lymphoma.

Methods: Data were retrospectively collected from medical records and the institutional database at Queen Elizabeth Hospital, Malaysia. The study included adult patients with lymphoma who underwent autologous stem cell transplantation (ASCT) between March 2016 and March 2024. Patient demographics, clinical characteristics, and treatment outcomes were extracted and analysed using the Statistical Package for the Social Sciences (SPSS) version 26.

Results: A total of 36 patients achieved adequate peripheral blood stem cell mobilization and subsequently underwent autologous stem cell transplantation (ASCT). Of these, 6 patients (17%) were transplanted in first complete remission (CR), 6 (17%) in second or third CR, and 24 (66%) in partial remission (PR). Conditioning regimens included BEAM in 31 patients and thiotepa-based regimens in 5. Among those transplanted in PR, 25% (n=6) converted to CR post-ASCT. Fourteen patients required additional therapy following transplantation: brentuximab vedotin maintenance (n=11), radiotherapy (n=2), or salvage chemotherapy for disease relapse. The 2-year overall survival (OS) rate was 75%, with a median follow-up of 13 months. The principal causes of mortality were disease progression and early relapse within six months post-transplant, while two patients experienced late relapse beyond this period.

Conclusions: Autologous stem cell transplantation (ASCT) remains integral to the management of aggressive lymphomas. For patients not achieving complete remission pre-transplant, post-ASCT therapies such as brentuximab vedotin (AETHERA trial) or radiotherapy may improve disease control and progression-free survival.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Challenging the Odds: A Case of Relapsed Hodgkin Lymphoma Post-Autologous Hematopoietic Stem Cell Transplantation

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Aims: Hodgkin lymphoma (HL) is a highly curable hematologic malignancy; however, a subset of patients experiences relapse even after high-dose chemotherapy and autologous stem cell transplantation (ASCT), which is considered the standard of care for relapsed or refractory disease. This case aims to present a case that exemplifies a highly refractory course of HL and the challenges in its management.

Methods: We present the case of a 24-year-old female diagnosed with classic Hodgkin lymphoma during pregnancy. She completed 12 cycles of ABVD chemotherapy followed by 16 cycles of Brentuximab vedotin. Despite this, a persistent left supraclavicular lymphadenopathy was noted. Interim PET-CT demonstrated partial metabolic response, showing regression of lymphadenopathies in the cervical, supraclavicular, parasternal, paratracheal, and axillary regions, along with resolution of a right lung base nodule. Mild FDG uptake in the nasopharynx and tonsillar regions was observed, likely reactive or inflammatory in nature. Given concerns for residual disease, the patient was offered intensified chemotherapy but initially declined. Instead, she received four cycles of pulsed dexamethasone, during which interval progression of nodal size was noted. She subsequently consented to two cycles of Br-ICE salvage chemotherapy while preparing for transplant. This was followed by high-dose BEAM (bendamustine, etoposide, cytarabine and melphalan) conditioning and autologous stem cell transplantation.

Results: Unfortunately, PET-CT performed on day +100 post-transplant showed progressive disease, indicating early relapse. Lenalidomide was initiated as post-ASCT therapy. A follow-up PET-CT revealed increasing size, number, and metabolic activity of cervicothoracic lymph nodes, with stable splenomegaly and newly identified FDG-avid infiltrates in the left lower lobe.

Conclusions: Relapse of Hodgkin lymphoma following autologous hematopoietic stem cell transplantation often carries a poor prognosis. This case highlights a highly refractory course of Hodgkin lymphoma, with limited response to multiple lines of therapy and early relapse following ASCT, emphasizing the critical need for novel and more effective treatment approaches. In this setting, palliative chemotherapy plays a crucial role in symptom control and disease burden reduction.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

CART CELL THERAPY BASED ON ALLO-HSCT IN A PATIENT WITH R/R DLBCL

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Background:
Patients with relapsed or refractory diffuse large B-cell lymphoma (R/R) DLBCL after autologous stem cell transplantation(ASCT) or autologous CART cell therapy have an extremely poor prognosis. Non-remission or recurrence of therapy may be related to genetic susceptibility genes in somatic germlines. Although the efficacy of allogeneic hematopoieticstem cell transplantation(allo-HSCT) is not ideal for diffuse large B-cell lymphoma, it can reduce the immune platform of patients to replace with donor type, and then undergo donor CART cell therapy, which may improve the prognosis of patients.

Aims:
To report long-term outcomes in patients treated with donor-derived CART cells after allo-HSCT to replace the patient's immune platform.

Methods:
Genetic susceptibility gene screening was performed for patients with DLBCL who had undergone ASCT or autologous CART cell therapy after recurrence. If they had genetic high-risk factors, allo-HSCT was performed. After changing the immune platform, donor CART cell therapy was performed to evaluate the efficacy, maintenance therapy could be performed after remission, and survival follow-up was performed.

Results:
Two female patients were treated in 2020 and have since continued to go into remission. They were 38 and 56 years old at the time of treatment in 2020 and had relapsed after receiving third-line or higher therapy including ASCT/autologous CART cell therapy prior to allo-HSCT. Genetic susceptibility gene tests for both showed more than 3 significant gene mutations identified by authoritative literature and 10 potentially harmful gene mutations identified by at least 2 software analyses. Both patients achieved long-term disease-free survival (DFS) of 5 years after allo-HSCT by donor CART cell therapy.

Conclusions:
The prognosis of R/R DLBCL after ASCT/ autologous CART is pessimistic, and the efficacy of allo-HSCT in the treatment of DLBCL is far less than that of B-ALL. Even though the prognosis of allo-HSCT is discouraging, the immune platform after allo-HSCT has changed to the donor type, and cell immunotherapy again may achieve good results and obtain long-term DFS.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Tisagenlecleucel Versus Axicabtagene Ciloleucel in DLBCL: A Real-World Safety and Survival Analysis

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Objective: CD19-directed chimeric antigen receptor (CAR) T-cell therapies have significantly altered the treatment landscape for relapsed or refractory diffuse large B-cell lymphoma (DLBCL). However, studies comparing the safety and effectiveness of tisagenlecleucel (tisa-cel) and axicabtagene ciloleucel (axi-cel) in real-world clinical settings remain relatively scarce. This study aimed to evaluate the differences in treatment outcomes between these two therapies in DLBCL patients using a large-scale real-world database.

Methods: This study employed a retrospective cohort design, analyzing de-identified electronic health records from the TriNetX US Collaborative Network (2017–2024). Study subjects were adult patients diagnosed with DLBCL who received either tisa-cel or axi-cel. To mitigate selection bias, we utilized 1:1 propensity score matching (PSM) to balance baseline characteristics between the two treatment groups, including demographics, comorbidities, prior treatment history, and laboratory data. Primary outcomes assessed were the risks of immune effector cell-associated neurotoxicity syndrome (ICANS) and cytokine release syndrome (CRS), as well as overall survival (OS). Secondary outcomes investigated post-treatment immune recovery by evaluating immunoglobulin G (IgG) levels across different ranges.

Results: After PSM, 148 matched pairs (296 patients total) were included for comparative analysis. Median follow-up was 1.94 years for the tisa-cel group and 1.71 years for the axi-cel group. Compared to axi-cel, patients receiving tisa-cel had significantly lower risks of ICANS (Hazard Ratio [HR], 0.42; 95% Confidence Interval [CI], 0.20–0.85) and CRS (HR, 0.56; 95% CI, 0.36–0.90). No statistically significant differences were observed between the groups in OS (HR, 1.23; 95% CI, 0.87–1.74) or IgG levels across various ranges. Multivariable analysis identified smoking status, diabetes, higher body mass index (BMI), multiple prior lines of chemotherapy, and elevated baseline C-reactive protein (CRP) levels as independent predictors influencing clinical outcomes.

Conclusion: This study, using real-world evidence, found that for DLBCL patients, tisa-cel demonstrated a more favorable safety profile compared to axi-cel, particularly in reducing the risks of ICANS and CRS, while offering comparable long-term survival benefits. The findings also highlight the importance of patient-intrinsic factors such as inflammatory markers (e.g., CRP), metabolic status (e.g., diabetes, BMI), and treatment history on CAR-T therapy prognosis. These results support the consideration of individual patient risk factors by clinicians when selecting CAR-T therapies to develop more personalized treatment strategies.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

Predictive Factors of Febrile Neutropenia and Severe Neutropenia in Patients with Diffuse Large B Cell Lymphoma (DLBCL) Receiving R-CHOP-21 Chemotherapy

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Aims: To determine the incidence of febrile neutropenia and severe neutropenia in patients receiving R-CHOP-21 regimen in the treatment of diffuse large B cell lymphoma (DLBCL). Additionally, to determine the predictive factors based on baseline characteristic of the patients including demographic, clinical and laboratory data.

Methods: We conducted a cohort retrospective analysis of 46 patients with newly diagnosed DLBCL receiving the first R-CHOP-21 chemotherapy without primary G-CSF prophylaxis in Wahidin Sudirohusodo Hospital from January 2023 – March 2025. The outcome of the study was the incidence and risk factors of febrile neutropenia and severe neutropenia after the first chemotherapy cycle.

Results: A total of 46 patients, the median age was 57 years old and 24 (52,2%) were female. The incidence of febrile neutropenia and severe neutropenia after the first chemotherapy cycle was 6 (13.0%) and 9 (19.6%), respectively. Bivariate analysis showed that aged 60 or above was the only significant risk factor of febrile neutropenia (*p=0.039*), whereas significant risk factors of severe neutropenia were ECOG PS ^{3} 2 (*p=0.015*), lower platelet count < 275.10^{3}/mL (*p=0.022*), and female sex (*p=0.023*). Multivariate analysis showed that male sex and increasing platelet count were associated with decreased risk of developing severe neutropenia {(Odds ratio, OR 0.084; 95%CI 0.009 – 0.826; *p value 0.034*); (Odds ratio, OR 0.076; 95%CI 0.008 – 0.739; *p value 0.026*), respectively}.

Conclusion: Severe neutropenia often occurs in DLBCL patients receiving R-CHOP-21 chemotherapy that can develop into febrile neutropenia. Thus, primary

prophylaxis with G-CSF should be considered especially in elderly, female patients, and patients with lower platelet count.

Poster Presentation

Lymphoma and Chronic Lymphocytic Leukemia

A CASE OF DIFFICULT PICC EXTUBATION IN A DLBCL PASTIENT case of difficult PICC extubation in a diffuse large B lymphoma (DLBCL) patient

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Background: Peripherally Inserted Central Catheter（PICC）are widely used in patients with long-term intravenous therapy and should be removed when the catheters expire or no longer need to be used. Generally, after X-ray, ultrasound and other auxiliary examinations confirm the situation of blood vessels and catheters, the removal is performed after carefully excluding contraindications, and there are still difficulties in extubation. Usually by hot compress, massage techniques to remove, if still can not be successfully extubation, need to interventional surgery and other invasive methods to remove.

Aim: The effectiveness of lidocaine injection in treating extubation difficulties caused by vasospasm was verified by a successful non-invasive extubation in a patient with PICC difficulty.

Method: The management of Diffuse large B lymphoma (DLBCL) patients with difficulty in extubation was studied retrospectively. The patient was a 50-year-old male who had been implanted with PICC catheter due to chemotherapy for more than 10 months. The PICC catheter needed to be removed after the end of treatment. The results of B-ultrasound and X-ray examination showed that the PICC catheter was complete, without thrombus, distortion and knotting, and no contraindications. After signing the informed consent of PICC extubation, the extraction began. The PICC specialist nurse slowly and gently extubated the catheter. Resistance increased at 10 cm, and the patient complained of slight pain. After placating the patient and giving the local hot compress for 15 minutes, and massaging at the same time, the catheter was extubed again, the resistance was not reduced, and the extubation failed again. A multidisciplinary bedside consultation was requested, and the surgical specialist determined that the patient had been diagnosed with vasospasm that prevented catheter removal. Consultation recommendation: Local infiltration of lidocaine anesthetizing vascular skin to achieve the purpose of relieving vasospasm and reducing resistance, infiltration anesthesia for 15 minutes, again try to extubation.

Result: According to the surgical consultation opinion, the sterile gauze was soaked with 1% lidocaine injection, applied to the puncture site and its surrounding area, and then extubation was attempted again for 15 minutes, the resistance was significantly reduced, the patient had no pain, the catheter was removed smoothly, the tube end was intact, and the patient had no discomfort after extubation. Interventional extubation is avoided, the pain of patients is reduced, and the cost of medical treatment is saved.

Conclusion: In this case, when it was difficult to relieve the spasm with conventional massage and hot compress, the patient was wet applied with sterile gauze soaked with lidocaine, and the contact infiltration anesthesia was used to effectively relieve the vasospasm and greatly reduce the possibility of lidocaine entering the blood vessel, thus reducing the risk of causing arrhythmia and other adverse consequences. The results also verified the effectiveness and safety of the method, and developed a new method to solve the difficulty of extubation caused by vasospasm.

Poster Presentation

Multiple Myeloma

Autologous Stem-Cell Transplant in First Remission or Later for Multiple Myeloma with Standard risk Cytogenetics – Does it Make a Difference for a Low-Middle Income Country?

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Aims:
Autologous stem cell transplant (ASCT) is a standard of care treatment for transplant-eligible multiple myeloma. There is a growing trend towards deferred ASCT, especially for standard-risk myeloma (SRMM) patients, in view of absence of survival benefit. There is paucity of literature from India which has compared ASCT in first-remission or later for patients with SRMM.
Primary objective was to determine median progression-free survival (PFS) and overall survival (OS) of SRMM patients transplanted in first-remission (CR1) vs later (CR2) cohorts. Secondary objectives were – to determine median PFS and OS of the whole cohort, and to compare the median PFS between CR1 vs CR2 cohorts for the following subgroups [Melphalan 200 mg/m^2 conditioning (Mel-200), hyper-diploid cytogenetics, ≥VGPR pre-ASCT, bone-marrow (BM) flow-cytometry MRD, and PET-CT MRD negative pre-ASCT].

Methods:
From 1st March 2007 to 31st December 2022, 176 patients underwent their first ASCT for MM. Amongst patients with available cytogenetics (n=150), 90 patients (60%) were SRMM. Patients without any high-risk cytogenetic abnormality [t(4;14); t(14;16); t(14;20); 17p deletion;1q gain/amplification or 1p deletion] were considered as SRMM.
BM MRD was evaluated using a highly-sensitive 13-color flow cytometry (sensitivity-0.001%) method. Any detectable MRD was defined as positive. Data was updated till 30th November, 2024. Survival and follow-up were calculated from date of ASCT.

Results:
90 patients with SRMM were analysed. Amongst these, 63 (70%) had undergone

ASCT in first remission, while 27 (30%) underwent at relapse. Median age of our cohort was 48 years (Range - 32-65 years) with a male predominance (n=65;72%). Median follow-up of the whole cohort was 60 months. Baseline characteristics of CR1 vs CR2 cohort are shown in **Table 1**. (*Table 1, attached at the time of submission, was deleted due to space limitations. —BCT Editorial Office*) Both groups were comparable for their baseline and pre-ASCT characteristics, except for ISS stage and disease status at ASCT. Amongst patients transplanted at relapse (n=27), 20 (74%) underwent ASCT after two lines of therapy, while 7 (26%) underwent transplant after 3 lines. Patients who were transplanted at relapse were less likely to attain PET-CT negativity (74% vs 50%; p=0.07). For CR1 vs CR2 cohorts, median PFS was not-reached (NR) vs 29 months (p=0.000), while median OS was NR for both cohorts (p=0.089) respectively. Median PFS and OS of the entire cohort was 80 months and NR, respectively. In subgroup analysis, median PFS was significantly better for CR1 cohort, even in subgroups of - Mel-200 (NR vs 29 months; p=0.001), ≥VGPR pre-ASCT (NR vs 28 months; p=0.000), BM MRD negative (81 months vs NR; p=0.07), PET-CT MRD negative (NR vs 33 months; p=0.000). Patients with hyperdiploidy had comparable PFS in CR1 vs CR2 (80 months vs NR; p=0.7).

Conclusions:

For standard-risk myeloma, transplantation in first-remission significantly improves the progression-free survival, over transplant at relapse. This difference persists even in patients who achieve a deep remission (≥VGPR, MRD negativity in BM and PET-CT) prior to ASCT, thereby justifying the importance of early transplant for our patients. While patients with hyperdiploidy appear to have equivalent outcomes irrespective of timing of ASCT, these numbers are small and prospective studies are needed to validate this finding.

Poster Presentation

Multiple Myeloma

Clinical Outcomes of Autologous Stem Cell Transplantation in Adults From a Resource-Constrained Tier-2 City in India: A 5-Year Real-World Experience Under a State-Sponsored Program

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Background:
Autologous stem cell transplantation (ASCT) represents a key therapeutic modality for hematologic malignancies such as multiple myeloma (MM) and relapsed/refractory lymphomas. While outcomes from established transplant centers in high-resource settings are well documented, real-world data from newly developed transplant centers in low- and middle-income countries (LMICs), particularly tier-2 Indian cities, remain sparse. These regions often face barriers including limited infrastructure, constrained human resources and infection control challenges. In 2019, our tertiary oncology center initiated a comprehensive transplant program supported by a government-funded scheme. This study presents the 5-year outcomes of ASCT performed in adult patients at our center, evaluating feasibility, engraftment kinetics, transplant-related mortality (TRM), and overall survival (OS).

Methods:
We conducted a retrospective analysis of adult patients (≥18 years) who underwent ASCT between August 2019 and February 2025. Majority of patients received treatment under a state-sponsored health insurance scheme covering the entire transplant process. Peripheral blood stem cells were mobilized with granulocyte colony-stimulating factor (G-CSF), with plerixafor added as needed. Conditioning regimens included high-dose melphalan for MM and the BEAM protocol for lymphoma. Key endpoints analyzed were demographic characteristics, stem cell yield, time to neutrophil and platelet engraftment, TRM (defined as death within 100 days post-transplant unrelated to disease progression), and OS.

Results:
A total of 88 ASCTs were performed over 5 years, of which 80% were funded by the

government scheme. MM accounted for 55 transplants, while 31 were for relapsed/refractory lymphoma. There was one case each of relapsed acute promyelocytic leukemia (APL) and germ cell tumor (GCT). The median age for MM patients was 55 years (range: 27–66), with a male-to-female ratio of 1.89:1. In lymphoma patients, the median age was 42 years (range: 20–60), with a male-to-female ratio of 1.5:1. Median CD34+ stem cell doses were 6×10^6/kg (MM) and 6.5×10^6/kg (lymphoma). For MM patients, median neutrophil and platelet engraftment occurred by day 10 and day 14, respectively. TRM was 1.8% (1/55), with OS at 31 months of follow-up reaching 87%. For lymphoma patients, median neutrophil and platelet engraftment occurred by day 11 and day 15, respectively. TRM was 9.6% (3/31), and OS at 29 months was 74%. The APL patient remained in remission at 13 months, while the GCT case relapsed at 5 months.

Conclusion:

Our experience highlights that autologous transplantation can be safely and effectively performed in a newly developed transplant unit within a tier-2 city, even amidst resource limitations. The encouraging survival outcomes and low transplant-related mortality achieved under a fully state-sponsored healthcare model reinforce the viability of expanding advanced hematologic care beyond metropolitan centers. This model not only bridges regional disparities in access to life-saving therapies but also demonstrates that with structured planning, appropriate training, and government support, high-quality transplant care is achievable in low- and middle-income settings.

Poster Presentation

Multiple Myeloma

Successful Haploidentical Stem Cell Transplantation in a Patient with Concurrent Multiple Myeloma and Primary Myelodysplastic Syndrome: A Case Report

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Aim: The coexistence of multiple myeloma (MM) and primary myelodysplastic syndrome (pMDS) is a rare and complex clinical phenomenon. Although MM and MDS are distinct hematologic disorders with different pathophysiologies, their simultaneous occurrence presents significant diagnostic and therapeutic challenges. Haploidentical stem cell transplantation (Haplo-SCT) has emerged as a promising therapeutic option, but its application in patients with both MM and MDS remains underexplored.

Method: We describe the case of a 41-year-old woman diagnosed with both MM and pMDS.

Result: The patient initially presented with symptoms consistent with MM, and further evaluation revealed concurrent pMDS. Due to poor response to chemotherapy and a high risk of progression, a decision was made to proceed with haploidentical stem cell transplantation. The patient underwent reduced-intensity conditioning (RIC) followed by haplo-SCT from a 9/10 HLA-mismatched daughter donor with post-transplant cyclophosphamide (PTCy). Care focused on preventing graft-versus-host disease (GVHD) and managing the dual hematologic conditions. Remarkably, the patient achieved complete remission of both MM and pMDS, with sustained engraftment and no signs of relapse after 30 months of follow-up.

Conclusion: This study highlights the potential of haplo-SCT as a feasible and effective treatment strategy in patients with coexisting hematologic malignancies, particularly when HLA-matched donors are unavailable. The successful outcome underscores the importance of considering haploidentical transplantation as a viable treatment option for patients with concurrent MM and MDS.

Poster Presentation

Multiple Myeloma

Beyond Hematologic Cure: Secondary Colon Cancer Emerging 13 Years After Autologous Stem Cell Transplantation

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Background:
Autologous hematopoietic stem cell transplantation (HSCT) is a well-established treatment modality for various hematologic malignancies, providing durable remissions and improved overall survival. However, as survival outcomes improve, long-term complications such as secondary solid tumors have become increasingly important. Secondary malignancies are driven by cumulative exposure to cytotoxic chemotherapy, radiation (in certain cases), chronic immune dysregulation, and other environmental or genetic factors. Among these, colorectal adenocarcinoma is an uncommon but clinically significant secondary cancer following HSCT. Despite its lower incidence compared to skin, thyroid, or lung cancers, colorectal cancer poses substantial morbidity if detected late, highlighting the need for vigilance in long-term HSCT survivors.

Case Presentation:
We report a case of a 55-year-old male, a long-term survivor of autologous HSCT performed 13 years ago for multiple myeloma. The patient presented with altered bowel habits over three months, characterized by intermittent constipation, changes in stool caliber, and lower abdominal discomfort. Physical examination was unremarkable, with no palpable masses or organomegaly. Laboratory studies were within normal limits, including complete blood count and tumor markers.
Colonoscopy revealed an ulceroinfiltrative mass lesion in the sigmoid colon, approximately 13 cm from the anal verge, with mucosal friability and contact bleeding. Histopathological analysis confirmed moderately differentiated adenocarcinoma. There was no personal or family history of colorectal cancer or hereditary cancer syndromes. Contrast-enhanced multislice computed tomography (MSCT) of the abdomen and pelvis demonstrated localized circumferential wall thickening of the sigmoid colon without evidence of distant metastasis, liver lesions, peritoneal involvement, or significant lymphadenopathy. The patient was diagnosed with non-metastatic sigmoid colon adenocarcinoma.
Systemic chemotherapy with the FOLFOX regimen (5-fluorouracil, leucovorin,

oxaliplatin) was initiated following standard treatment guidelines. The patient tolerated chemotherapy well, with no severe adverse events. Follow-up clinical and imaging assessments demonstrated stable disease, with no signs of progression.

Discussion:

Secondary solid tumors, including colorectal cancer, represent a serious late effect of HSCT. The latency period between HSCT and the development of secondary malignancies can exceed a decade, as seen in this case. The pathogenesis involves cumulative genotoxic effects from prior high-dose chemotherapy, persistent alterations in immune surveillance, and possible synergistic effects with aging and environmental risk factors.

While the management of secondary colon cancer generally follows standard oncologic protocols, special considerations are required in HSCT survivors. Factors such as prior organ toxicity, marrow reserve, and increased susceptibility to treatment-related adverse effects must be carefully evaluated. Current evidence does not indicate a distinct biological difference between HSCT-related and sporadic colorectal cancers, but the need for tailored surveillance strategies remains a topic of ongoing research.

Conclusion:

This case illustrates the occurrence of secondary sigmoid colon adenocarcinoma as a late complication of autologous HSCT, emerging 13 years post-transplant. It underscores the importance of longterm follow-up and routine colorectal cancer screening in HSCT survivors. Early diagnosis and multidisciplinary management are essential to ensure favorable outcomes in this high-risk patient population.

Poster Presentation

Myelodysplastic Syndromes

Anorectal Infiltration of a Therapy-Related Myeloid Neoplasm: A Previously Unreported Presentation

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Aims: To report a unique case of extramedullary localization of a PARP inhibitor-related myeloid neoplasm (MN-pCT) involving the anal canal and perianal skin, a localization never described to date.

Methods: We reported a case of a 65-year-old female patient with a known history of somatic BRCA1-mutated ovarian carcinoma, in remission after a treatment with platinum-based chemotherapy and PARP inhibitor from 2021 to 2023. After developing transfusion-dependent pancytopenia in early 2024, bone marrow studies revealed a therapy-related myelodysplastic syndrome (t-MDS) with complex karyotype, TP53 and PPM1D mutations. During hematologic follow-up, she presented with a painful perianal lesion initially managed as an abscess. Surgical biopsies from the anal canal and adjacent perianal tissue were analyzed histologically and immunohistochemically.

Results: Histologic examination of the anal canal and perianal skin revealed ulceration and necrosis associated with a dermal and submucosal infiltrate of atypical blastoid cells. Immunohistochemistry showed positivity for CD68, CD117, myeloperoxidase (MPO), CD43, p53, and partial CD15 expression, consistent with myeloid lineage. There was no evidence of leukemic transformation in the bone marrow at the time. The patient was then treated with Azacitidine which was stopped due to significant thrombocytopenia. A transformation of the myelodysplastic syndrome with more than 20% of blasts in peripheral blood occurred 6 months after the diagnosis of myeloid sarcoma. The patient died concomitantly from a probable cerebral hemorrhage favored by thrombocytopenia. A literature review identified only a limited number of extramedullary MN-pCT cases as myeloid sarcoma, primarily involving the skin, scalp, leptomeninges, and mediastinum - but none involving the anorectal region.

Conclusions: This case represents the first documented instance of MN-pCT involving the anal canal and perianal skin, expanding the recognized anatomical spectrum of myeloid sarcoma. The clinical presentation mimicked a rectovaginal

fistula or Crohn-like inflammation, highlighting the importance of early histopathological and immunophenotypic evaluation. In patients with a history of MN-pCT, any atypical ulcerative or inflammatory lesion should prompt consideration of myeloid infiltration. Awareness of this rare presentation is critical for timely diagnosis and management.

Poster Presentation

Conditioning Regimens

Optimization of TBI-based Myeloablative Conditioning Regimen Incorporating ATG and PTCY in Adult Patients with Acute Lymphoblastic Leukemia Undergoing Allogeneic Hematopoietic Cell Transplantation: Historical Comparison with TBI/CY

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AIMS: Historical myeloablative conditioning (MAC) regimen for hematopoietic cell transplantation (HCT) has been developed with total body irradiation (TBI) first, and additional cyclophosphamide (CY) was introduced for graft-versus-host disease (GVHD) reduction with its anti-inflammatory property. Also in adult patients with acute lymphoblastic leukemia (ALL), TBI/CY is the most widely used conditioning regimen, but it still shows high relapse rate over than 30%, which is similar to that of several reduced toxicity conditioning (RTC) regimens, prompting the need for alternative strategies. As we previously experienced significantly lower long-term relapse rate in cord blood transplantation following TBI, high-dose cytarabine, and fludarabine (TAF) regimen, we are now conducting observational prospective study using this new MAC regimen with refined GVHD prophylaxis.

METHODS: New MAC regimen TAF (TBI 1200 cGy, cytarabine 9g/BSA, and fludarabine 150mg/BSA) was used since Nov 2022, and 33 patients were enrolled until Dec 2023 with a median follow-up duration of 12.2 months (range 6.2-20.8 months). In this study, we compared HLA-matched donor transplants in CR1 who were treated with TAF regimen (n=26) and historical TBI/CY (n=87, from 2018 to 2022). For MUD transplants, GVHD prophylaxis was modified using a combination of ATG 2.5mg/kg and post-transplantation cyclophosphamide (PTCY) 30mg/kg for 2 days in TAF regimen, while ATG 2.5mg/kg was used in historical TBI/CY regimen.

RESULTS: Estimated 1-year DFS was superior after TAF than TBI/CY (91.1% vs. 66.6%, p=0.0182) with significantly lower 1-year relapse rate (5.1% vs. 25.3%, p=0.0219). However, 1-year OS (88.1% vs. 89.6%, p=0.727) and NRM rates (2.3% vs. 3.8%, p=0.665) were similar between two groups. In MSD-HCT, the incidence of grade 2-4 acute GVHD was similar between TAF and TBI/CY (29.9% vs. 37.1%) but moderate to severe chronic GVHD was more frequent as TAF was without ATG and

PTCY (51.8% vs. 22.9%, P=0.013). However, in MUD-HCT, the incidence of grade 2-4 acute GVHD was also similar (55.6% vs. 55.8%) but chronic GVHD was less frequent as TAF was with ATG and PTCY (22.2% vs. 44.2%, P=0.139).

CONCLUSIONS: Our data suggested TAF consequently showed lower relapse and better DFS than historical TBI/CY in adult ALL but showed higher incidence of chronic GVHD in MSD-HCT without both pretransplant-CY and PTCY. Opposite MUD-HCT results showed PTCY may offer benefit in lowering chronic GVHD for TBI containing regimens.

Poster Presentation

Conditioning Regimens

Comparative Outcome Analysis of Non-TBI versus TBI-based Reduced Toxicity Conditioning Regimens in Adult Patients with Acute Lymphoblastic Leukemia

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Aims: The role of total body irradiation (TBI), used as part of the pretransplant conditioning regimen, is still arguable in adult patients with acute lymphoblastic leukemia (ALL). Here, we are going to conduct a comparative analysis in terms of survival outcomes, incidence of GVHD and toxicity profile between TBI-based vs. non-TBI based reduced-toxicity conditioning (RTC) regimens.

Methods: We tried to evaluate a newly optimized RTC regimen which consisted of fludarabine 150 mg/m^2, melphalan 100 mg/m^2, and low-dose TBI 400-800 cGy (TBI-based regiemen) in combination with GVHD prophylaxis using ATG/PTCy in 96 adult patients with ALL undergoing allo-HCT in CR1. We compared this TBI-based new regimen to 256 historical controls receiving non-TBI based fludarabine 150 mg/m^2 plus busulfan 9.6mg/kg or melphalan 140mg/BSA with ATG. Key endpoints included disease-free survival (DFS), overall survival (OS), relapses, non-relapse mortality (NRM).

Results: After both transplantation (non-TBI vs. TBI-based), neutrophil (12 vs. 13 days) and platelet (13 vs. 14 days) recovery was similar, but diarrhea, nausea, and oral mucositis was more frequent in TBI-based regimen. The incidence of acute GVHD II-IV was not different (39.5% vs. 45.9%, p=0.152), but acute GVHD III-IV was higher in TBI-based regimen (11.3% vs. 19.8%, p=0.032). The incidence of moderate to severe chronic GVHD was not different (26.6% vs. 19.6%, p=0.142). At one year evaluation, cumulative incidence of relapse was lower in TBI-based regimen (29.9% vs. 16.7%, p=0.025), but NRM was similar (15.7% vs. 12.9%, p=0.976). TBI-based regimen had superior DFS (70.4% vs. 54.4%, p=0.071) and a trend toward improved OS (80.7% vs. 66.0%, p=0.291). Final multivariate analysis revealed DFS was poor in 40 years or older and HCT-CI with 3 or higher, whereas TBI-based regimen showed better DFS (HR 0.58; 95%CI 0.3-0.9, p=0.034) with significantly lower incidence of relapse (HR 0.35; 95%CI 0.2-0.7, p=0.002).

Conclusions: Our newly optimized TBI with ATG/PTCy combination showed improved DFS compared with non-TBI-based regimens showing similar GVHD incidences than expected.

Poster Presentation

Conditioning Regimens

LACE - an Effective Conditioning Regimen for Primary Central Nervous System Lymphoma (PCNSL) Patients Undergoing Autologous Transplant

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Background: First-line treatment of PCNSL involves high-dose methotrexate-based chemoimmunotherapy. Patients achieving response should receive consolidation with high-dose chemotherapy followed by ASCT, which improves long-term outcomes (supported by 2 RCTs). BEAM (BCNU, etoposide, cytarabine, melphalan) and thiotepa-based regimens are commonly used. BEAM has high toxicity; thiotepa-based regimens show day-100 TRM of 2–7%. LACE (lomustine, cytarabine, etoposide) is our center's preferred regimen, showing outcomes comparable to BEAM but with significantly lower toxicity and NRM. All LACE agents penetrate the CNS effectively, making it a safer and efficacious alternative for conditioning in PCNSL transplants.

Aims and Objectives: - To assess the outcomes in patients with PCNSL undergoing ASCT with LACE regimen. The primary objective was to determine the 2-year progression-freesurvival (PFS). The secondary objectives were to assess the regimen-related toxicities, and engraftment kinetics.

Methods: This single-centre retrospective cohort study includedpatients of PCNSL who received LACE as the conditioning regimen from January 2008 to December 2023. LACE regimen was given as lomustine200 mg/m2/dayon d-7, etoposide 1000mg/m2/dayon d-7, cytarabine 2000 mg/m2/dayon d-6 and d-5 and cyclophosphamide 1,800mg/m2/dayon d-4 to d-2. Mesna was given at 1800 mg/m2 BD on day-4 to day-2. Antiviral prophylaxis with acyclovir and antifungal prophylaxis with voriconazole was given to all patients.

Results: Nine patients (median age 49 years) received LACE. Eight patients received the CALGB MTR protocolinduction (3 of these with lenalidomide replacing temozolamide as a part of a clinical trial), while 1 patient received the De-Angelis protocol. Seven patients received cytarabine +/- etoposide after induction because of delay in transplant. Stem cell mobilization was done using G-CSF + plerixafor in5 patients and cytarabine chemo-mobilization in 4. At the time of transplant, 8

patients were in CR (7 in CR1 and 1 in CR3), and 1 patient was in partial remission. One patient required an unplanned omission of cyclophosphamide because of development of sepsis during conditioning. Post-transplant, none had grade III or more mucositis and only 1 had grade III diarrhoea. The median day of WBC and platelet engraftment was 11 (range 9-17) and 13 (range 10-22) respectively. With a median follow-up of 11.1 months, the 2-year PFS was 50%. When only patients transplanted in CR-1 were considered (n=7), the 2-year PFS was 66%. Three patients relapsed and 1 patient died of an unrelated cause. There was no transplant-related mortality.

Discussion: Thiotepa-based conditioning regimens have a similar 2-year RFS of about 67–73%, but have a TRM of about 2-7%. BEAM regimen is associated with a lower 2-year RFS of around 40%, due to suboptimal CNS drug delivery. LACE regimen haslow toxicityand low TRM with reasonable PFS.

Conclusions: LACE regimen in PCNSL is a promising regimen thatshows comparable outcomes with other conditioning strategies (especially in patients in CR1), with an advantage of low regimen-related toxicity and TRM. This regimen needs to be explored in larger studies.

Poster Presentation

Conditioning Regimens

Clinical Study of Second Allogeneic Hematopoietic Stem Cell Transplantation in Treating 60 Patients with Relapsed Acute Myeloid Leukemia Post-Transplantation

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Objective:
To evaluate the efficacy and safety of second allogeneic hematopoietic stem cell transplantation (second HSCT) in treating patients with relapsed hematologic malignancies post-transplantation.

Methods:
A retrospective analysis was conducted on 60 acute myeloid leukemia (AML) patients who underwent second HSCT at Beijing Gaobo Boren Hospital from October 2020 to December 2023.

Results:
Among the 60 patients, 33 were male and 27 were female, with a median age of 29 years (range: 1–66) at the time of second HSCT. Age distribution: 1–18 years (14 cases, 23.3%), 19–40 years (35 cases, 58.3%), 41–60 years (10 cases, 16.7%), and >61 years (1 case, 1.7%). Fusion gene positivity was observed in 26 cases (AML1-ETO: 7, BCR-ABL1: 1, MLL-related: 8, NUP98-related fusions: 3, others: 7), and chromosomal karyotype abnormalities were detected in 33 cases. Pre-transplant disease status: complete remission (CR) in 24 cases, partial remission (PR) in 13, and no remission (NR) in 23. Conditioning regimens: BU/CLAG or FLAG-based (18 cases) and TBI/FLAG or CLAG-based (42 cases). All patients received donor switches: haploidentical transplants (48 cases, including 29 related and 9 non-related) and matched unrelated donor transplants (12 cases). One patient died of intracranial hemorrhage, and two died of multi-organ failure without neutrophil engraftment; the remaining 57 achieved neutrophil engraftment. Platelet engraftment was achieved in 55 cases (88.6%). Acute GVHD (aGVHD) occurred in 15 cases (25%: grade I-II in 7 [11.6%], grade III-IV in 8 [13.4%]), and chronic GVHD (cGVHD) developed in 20 cases (33.3%). Median follow-up post-second HSCT was 15 months (range: 1–47). Day-100 transplant-related mortality (TRM): CR group (16.6%, 4/24), PR group (7.7%, 1/13), NR group (30.4%, 7/23). One-year overall

survival (OS) rates: CR group (68.42%), PR group (66.67%), NR group (59.09%), with intergroup P=0.5312 (P>0.05). OS rates by conditioning regimen: Bu-based (44.4%, 95% CI: 21.5%–67.4%) and TBI-based (78.6%, 95% CI: 66.2%–91.0%), P=0.0214 (<0.05). One-year cumulative relapse rates: TBI group (19.05%) and BU group (22.22%), P>0.05.

Conclusion:

For AML patients, modifying the conditioning regimen during second HSCT improves post-transplant survival but does not significantly affect the one-year cumulative relapse rate.

Poster Presentation

Conditioning Regimens

Experience of haploidentical allo-HSCT in a patient with a high MFI donor-specific HLA antibody using desensitizing therapy

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Background:
Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is currently an obligatory part of therapy for a number of hematological diseases. When selecting a blood stem cell donor, preference is given to fully compatible related or unrelated donors, however in some cases this is not possible and haploidentical donors are considered.
In addition to a higher risk of developing GVHD, haploidentical transplantation is characterized by a large number of cases of graft failure, one of the predictors of which is a high MFI (≥ 5000) donor-specific HLA antibody (DSA). In such cases, due to lack of choice, desensitizing therapy is used before conditioning to reduce the titer of DSA.

Aims:
Our purpose was to demonstrate positive experience of haploidentical hematopoietic stem cell transplantation in a patient with high MFI DSA using desensitizating therapy.

Methods:
We present a case of successful haploidentical allo-HSTC in a patient with high MFI DSA using desensitizing therapy (rituximab 375mg/m^2 iv day -14 (after plasmapheresis); bortezomib 1,4 mg/m^2 sc days -18, -14, -11, -8; mycophenolate mofetil 30 mg/kg (over 2000 mg) po days -18 to +4; plasmapheresis №5 (1 CBV) days -18, -16, -14, -1, 0) and Flu180+Bu8 redused intencity conditioning regiment.

Results:
A 36-year-old female with alpha-beta hepatosplenic T-cell lymphoma achieved remission after five courses of chemotherapy using the ESGAP regiment. It is known that hepatosplenic T-cell lymphoma has the worst prognosis of the non-Hodgkin

lymphomas. The best way received a long overall survival and event-free survival is an allo-HSCT. One fully compatible donor was found in the registry, but unfortunately, at the stage of collecting stem cells, he refused to donate, so a haploidentical donor brother was chosen as the donor. During additional examination, the patient was found to have a high MFI DSA (10700) to the donor's HLA-A (02:01:01). We applied desensitization therapy before starting the Flu180+Bu8 conditioning regimen. Serum immunoglobulin G level was used as an indicator of the effectiveness of the therapy (7.16 g/l before and 3.72 g/l after). Engraftment was confirmed on day +15 of allo-HSCT. In the early post-transplant period, a number of viral infectious complications developed: CMV infection, hemorrhagic cystitis associated with BKPyV, orthopneumovirus infection of the upper respiratory tract.

Conclusion:

The use of desensitizing therapy makes it possible to perform allo-HSCT in patients with high MFI DSA, reducing the risk of graft failure. However, it should be taken into account that the inevitable depletion of humoral immunity can lead to an increase risk of infectious complications.

Poster Presentation

Conditioning Regimens

Early Outcomes of TBI- vs Thiotepa-Based Conditioning in Haploidentical Stem Cell Transplant: A 1-Year Comparative Analysis in Single Major Transplant Centre in Malaysia

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Aim:
Haploidentical stem cell transplantation (HaploSCT) has made HSCT to be more accessible to patients in providing to cure in haematological malignancies. Conditioning regimen choice significantly influences outcomes in HSCT. Total body irradiation (TBI) and thiotepa-based (TT) regimens are commonly employed, but comparative survival data remain limited. This study compares early outcomes between TBI-based and thiotepa-based conditioning regimens in terms of overall survival (OS), progression-free survival (PFS), and GVHD-free, relapse-free survival (GRFS) within the first year post-transplant.

Methods:
This retrospective cohort study included 46 patients undergoing haplo-HSCT in Ampang Hospital Malaysia from January 2015 to December 2024, with last follow up date on 30th April 2025. Data was collected from the electronic medical records. All TBI-containing regimens were included under the TBI-based group. Six patients received 2 Gy TBI, these were combined with high-dose chemotherapy (e.g., Flu/Bu) and classified as myeloablative by institutional standards. Median follow up in both cohorts were 4 months with longer follow-up duration in the TT group (0.31 to 64.62 months in TBI vs 1.41 vs 107.99 months in TT). Due to patient transfers to own centers, survival analysis was limited to 1 year to ensure data integrity. Kaplan-Meier analysis was used to estimate 100-day and 1-year overall survival (OS), relapse free survival (RFS), and GVHD free, relapse free survival (GRFS). Multivariate analysis to assess risk factors associated with OS, RFS and GFRS, using Cox regression.

Results:
At 100 days post-transplant, though was not statistically significant, OS and PFS were similar, 60.9% and 69.6% respectively for TBI and TT groups (p value 0.178,

0.198 and 0.720 for OS, RFS and GFRS respectively). GRFS was higher in the TT group (39.1% vs 42.5%). By 1 year, TT-based regimens showed better outcome than TBI: OS (55.8% vs 42.4%), PFS (48.2% vs 29.0%) and GRFS (32.6% vs 8.7%). Multivariate Cox regression analyses showed TT-based conditioning was consistently associated with significantly improved outcomes (OS: HR 0.234, p=0.013; PFS: HR 0.250, p=0.018; GRFS: HR 0.345, p=0.011). Non-ALL diagnoses (MPAL, MDS, AML) were independent predictors of worse outcomes, with hazard ratios ranging from 3.3 to 7.2 (p<0.05). GVHD prophylaxis with Post-transplant cyclophosphamide 100 mg was significantly associated with higher mortality (lower OS) and poorer PFS (HR 9.82 and 10.12, p=0.041 and 0.039, respectively). Sex and disease status at transplant were not significantly associated with outcomes in any model. In terms of other complications after HaploSCT, sinusoidal obstructive syndrome (SOS), it was seen only in TT-based group (n=4, p=0.036), while other complications such as thombotic microangiopathy, infections, graft failure, engraftment syndrome and CMV reactivation.

Conclusion:

Within the first year post-transplant, thiotepa-based conditioning demonstrated superior outcomes across OS, PFS, and GRFS metrics compared to TBI. These findings support the consideration of thiotepa-based regimens in haploidentical HSCT, especially in settings prioritizing early event-free survival.

Poster Presentation

Conditioning Regimens

Unveiling the Outcomes with BEAM Conditioning and Autologous Stem Cell Transplant in Relapsed/Refractory Lymphoma

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Aims: Salvage chemotherapy followed by autologous stem cell transplantation (ASCT) in responders remains the standard of care for relapsed/refractory (R/R) lymphoma. About 20-30% of patients with non-Hodgkin lymphoma (NHL) and 15% of Hodgkin lymphoma (HL) patients relapse after initial therapy. Despite the debate over the choice of the conditioning regimen, BEAM with carmustine (BCNU), etoposide, cytarabine, and melphalan continues to be widely used and our study was designed to analyse the outcomes of ASCT in R/R lymphoma with BEAM conditioning.

Methods: This was a retrospective, single centre study of R/R lymphoma patients who received salvage chemotherapy followed by ASCT over a period of three years. Demographic data, lymphoma type, stage, initial and salvage treatment information were noted. Post ASCT patients were regularly followed up at monthly interval for 3 months and then every 3 monthly. PET CT scan was done on Day 100 of ASCT to document response. Kaplan-Meier analysis was used to determine overall survival (OS) and relapse-free survival (RFS). The RFS in patients who underwent ASCT was also compared to those who did not undergo ASCT after the first salvage chemotherapy.

Results: A total 50 patients had R/R lymphoma of which of 30 patients underwent ASCT, with relapse in 23 patients (76.6%) and with refractory disease in 7patients (23.3%) (n=7). The median age was 35years (range,14-63) with male to female ratio of 2:1. NHL and HL patients constituted 50% each. The median treatment free interval in relapsed patients was 16 months (range, 3-192). All patients received BEAM as conditioning regimen. Median stem cell yield was 6×10^6/kg (range, 2.95-19.7). Median time to engraftment was 9 days (range, 7-11). The most common grade3/4 toxicities seen were mucositis in 8patients (26.6%) and diarrhoea in 5 patients (16.6%). One patient (3.33%) died during ASCT on day 10 due to gram negative sepsis. In the remaining 29 patients, day100 PET CT showed complete response in 25patients (86.2%), partial response in 2 patients (6.9%) and

progressive disease in 2 patients (6.9%). At a median follow up of 30months, RFS was 70% versus 20% (p=0.002) in patients who did and did not undergo ASCT respectively. And OS was 80% in the transplant group.

Conclusions: ASCT plays an indispensable role in R/R lymphoma. BEAM is a conversant conditioning regimen for the transplant physicians showing promising efficacy and tolerability.

Poster Presentation

Graft-versus-host Disease

Precision Timing and Dosing of Preemptive add-on Prophylactic Medication Against Severe Acute Graft-Versus-Host Disease: A Prospective Interventional Trial of a Machine Learning Model

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Background:
Allogeneic hematopoietic stem cell transplantation (allo-HSCT) has become the primary treatment method for some hematological diseases. The use of machine learning to integrate big data for predicting severe acute graft-versus-host disease (aGVHD) may provide a new path towards understanding immune complications after transplantation. We have developed a dynamic forecasting model for severe aGVHD, termed the 'daGOAT model', achieving an AUROC score of more than 0.78.

Aims:
To evaluate the efficacy and safety of ruxolitinib for prophylactic therapy of adult patients who are predicted to have a high risk for developing severe aGVHD by the daGOAT model.

Methods:
This is a prospective single-arm historical-control clinical trial. Adult patients receiving human leukocyte antigen (HLA)-mismatched allogeneic hematopoietic stem cell transplantation (allo-HSCT) will be enrolled after October 1, 2022. The daGOAT model will dynamically predict the risk for severe aGVHD daily from day 17 to day 23 after transplantation, and medication will be adjusted according to the predicted risk. **Model-predicted high-risk patients (HR)**: ruxolitinib 5mg bid po until at least day 60 post-transplant and terminated after day 100. If severe hematological signs occur such as when there is severe neutropenia (<0.1×109/L), ruxolitinib can be used at half dose or discontinued as appropriate, and can continue to be used after hematology recovery. Model-predicted moderate-risk patients(MR): ruxolitinib 2.5mg bid p po until at least day 60 post-transplant and terminated after day 100. If severe hematological signs occur such as when there is severe neutropenia (<0.1×109/L), ruxolitinib can be used at half dose or

discontinued as appropriate, and can continue to be used after hematology recovery. Model-predicted low risk(LR): regular aGVHD prophylactic regimens. Patients will be followed up at days 14, 28, 42, 60, 90, 180, 270, 360 and 540 after transplantation; data on infection, relapse, survival, and quality of life will be collected. Patients received HSCT from 2020/12/1 to 2021/12/31 were used as historical controls.

Results:
During 2023/1/17 to 2023/12/31, 44 patients were enrolled and completed the prediction. According to the model results from day 17 to day 23 after transplantation, there were 6 HR patients (14%), 20 MR patients (45%) and 18 LR patients (41%). A total of 3 cases of severe aGVHD occurred, with an incidence of 7%, which was significantly lower than that of historical controls (20%). Among them, 1 case of severe aGVHD in HR patients (17%) was significantly lower than that in the same group of historical controls (50%), 0 cases of severe aGVHD in MR patients (0%) was significantly lower than that in the same group of historical controls (30%), and 2 cases of severe aGVHD in low-risk patients (11%) were slightly higher than that in the same group of historical controls (6%).

Conclusion:
The daGOAT model is effective and safe for predicting adult patients at high risk of severe aGVHD. This is the interim report of the project, which needs to be supported by further patient data.

Poster Presentation

Graft-versus-host Disease

CD3+ T Cell Counts in Stem Cell Grafts and Their Association with Graft-Versus-Host Disease: A Retrospective Analysis

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Aims:
To assess the association between CD3+ T cell counts in allogeneic stem cell grafts and the incidence of acute and chronic graft-versus-host disease (GVHD) in patients undergoing hematopoietic stem cell transplantation (HSCT).

Methods:
We retrospectively analyzed 31 patients undergoing allogeneic HSCT (July 2019–Jan 2025) at our institution. Diagnoses included acute myeloid leukemia (AML, n=13), severe aplastic anemia (n=6), and others. Donors were matched related (MRD, n=17), haploidentical (Haplo, n=12), or mismatched related (MMRD, n=2). CD3+ T cell counts in peripheral blood or bone marrow grafts were measured by flow cytometry. Median CD3 dose was 1.54 x 10^8/kg (range: 0.62–3.93 x 10^8/kg); median CD34 dose was 7 x 10^6/kg (range: 4.3–10 x 10^6/kg). Primary outcomes were acute GVHD (grade II–IV) and chronic GVHD incidence. Secondary outcomes included overall survival (OS).Acute GVHD (aGVHD) was graded per MAGIC criteria, and chronic GVHD (cGVHD) per NIH criteria. Logistic regression and Fisher's exact test were used to assess GVHD risk, and Kaplan-Meier estimates evaluated OS, adjusting for donor type and conditioning regimen.

Results:
Acute GVHD occurred in 18 patients (58.1%), with grades II–IV in 13 (41.9%), primarily affecting gut and liver. Chronic GVHD developed in 8 patients (25.8%), with skin, liver, gut, oral, or eye involvement (mild to severe). Median CD3 dose was 1.62 x 10^8/kg in aGVHD cases vs. 1.31 x 10^8/kg in non-aGVHD (p=0.42, logistic regression). For cGVHD, median CD3 dose was 1.76 x 10^8/kg vs. 1.48 x 10^8/kg in non-cGVHD (p=0.38). Logistic regression showed no significant association between CD3 dose and aGVHD (OR: 1.14, 95% CI: 0.88–1.48, p=0.32) or cGVHD (OR: 1.12, 95% CI: 0.83–1.50, p=0.46). Fisher's exact test indicated a non-significant trend for higher aGVHD incidence with CD3 doses >2 x 10^8/k (60% vs. 38.9%, p=0.29)

and cGVHD (33.3% vs. 22.7%, p=0.67). At 1-year post-transplant, 19patients (61.3%) were alive. Kaplan-Meier estimates showed 1-year OS of 61.3% (95% CI: 42.0–76.1%), with no significant association with CD3 dose (p=0.48, log-rank test). Haploidentical transplants had lower OS (50.0% vs. 70.6% for MRD, p=0.26). Deaths (n=12) were due to GVHD (n=3), relapse (n=2), or other causes (n=7).

Conclusion:

CD3 dose in the stem cell graft was not significantly associated with aGVHD or cGVHD incidence, severity, or OS in this cohort. A trend toward increased aGVHD and cGVHD risk with higher CD3 doses warrants further investigation. Larger, multicenter studies are needed to elucidate the role of CD3 dose in optimizing HSCT outcomes.

Poster Presentation

Graft-versus-host Disease

Correlation Between Ferritin Levels, Trends, and GVHD Risk and Final Outcome After 1-Year Follow-Up: A Single-Centre Study

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Aims:
Elevated serum ferritin, as an acute phase reactant, may increase graft-versus-host disease (GVHD) risk and adversely affect outcomes in allogeneic hematopoietic stem cell transplantation (HSCT). This study evaluated the correlation between pre-transplant ferritin levels, post-transplant ferritin trends, GVHD incidence (acute and chronic), and 1-year outcomes (overall survival and relapse) in HSCT patients at a tertiary care center.

Methods:
A retrospective study analyzed 29 patients (19 males, 10 females, median age 40 years) who underwent allogeneic HSCT (12 MRD, 15 haploidentical, 1 syngeneic, 1 MUD) for hematological disorders (62% AML, 14% aplastic anemia, others) between January 2020 and September 2024. Pre-transplant ferritin levels were categorized as low (<1000 ng/mL), moderate (1000–2500 ng/mL), or high (>2500 ng/mL). Ferritin levels were monitored at D0, D+14, D+28, D+56, D+90, D+180, and 1 year. Data on acute and chronic GVHD (stage/organ) and 1-year outcomes (survival, relapse, death) were collected. Logistic regression assessed GVHD risk, and Kaplan-Meier estimates evaluated survival.

Results:
Of 29 patients, 23 had pre-transplant ferritin data (median: 643 ng/mL). Low, moderate, and high ferritin groups included 12, 6, and 4 patients, respectively. At 1-year follow-up, 14 patients (48.3%) were alive, 10 (34.5%) died, and 5 (17.2%) relapsed. High pre-transplant ferritin (>2500 ng/mL) was associated with higher mortality (50% vs. 33.3–40% in moderate/low groups). GVHD occurred in 15 patients (51.7%), with 12 (41.4%) experiencing acute GVHD (mostly stage 1–2 liver/gut/skin) and 5 (17.2%) chronic GVHD. High pre-transplant ferritin patients had a GVHD incidence of 50% (including one severe stage 4 gut/liver case), compared to 33.3% in the moderate group and 58.3% in the low group. Post-transplant ferritin trends showed that patients with persistently high or rising

ferritin levels (e.g., peaking at 19107 ng/mL by D+28/D+90) had more severe or chronic GVHD, while declining ferritin levels (e.g., from 2169 ng/mL at D0 to 328 ng/mL at 1 year) correlated with milder GVHD and better survival. No significant correlation was found between ferritin and relapse due to limited cases.

Conclusions:
High pre-transplant ferritin levels (>2500 ng/mL) and persistently elevated or rising post-transplant ferritin were associated with increased GVHD risk and higher mortality at 1-year post-HSCT, suggesting ferritin as an acute phase reactant linked to adverse outcomes. These findings support pre- and post-transplant ferritin monitoring to mitigate GVHD and improve survival. Larger studies are needed to confirm these trends and explore relapse associations.

Poster Presentation

Graft-versus-host Disease

Plasma exchange combined with bilirubin adsorption for the treatment of a patient with severe hepatic GVHD after second transplantation for acute myeloid leukaemia

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Aim:
The combination of plasma replacement and bilirubin adsorption therapy has been shown to be a highly effective treatment for reducing bilirubin levels in blood, alleviating symptoms of jaundice, and reducing the burden on the liver. The therapy has been observed to have a relatively low rate of side effects and can be repeated multiple times.In this study, we will be summarising the effects and treatment experience of plasma replacement combined with bilirubin adsorption therapy in a patient with acute myeloid leukaemia who presented with severe hepatic GVHD after a second allogeneic haematopoietic stem cell transplantation.

Methods:
Patient female, 13Y, acute myeloid leukemia with TEL-CCDC126 fusion gene diagnosis is clear, after the second transplantation of bone marrow flow relapse, in order to control the primary disease, DLI back infusion combined with targeted drug therapy, the primary disease obtained complete remission, but quickly appeared severe hepatic GVHD, the total bilirubin quickly rose from 39. 1umol / L to 637umol / L, given anti-GVHD and hepatoprotective drugs were ineffective, and total bilirubin rose to 735umol/L on 2024-10-8. In order to control the elevated bilirubin as soon as possible, further control the exacerbation of liver damage, and avoid hepatic encephalopathy, the feasibility of plasma exchange combined with bilirubin adsorption was considered after the consultation of the Department of Hepatology.On 2024-10-08/2024-10-11/2024-10-15, the TPE model of the COBE Spectra hemocyte separator and external Kenfan bilirubin adsorption column BS330, three plasma exchange combined with bilirubin adsorption treatments were performed. Strict aseptic technique was used in connecting the patient's venous access (femoral vein) and the patient was monitored cardiologically throughout. To prevent adverse effects, the patient was given calcium gluconate solution orally. The volume of plasma passed through the adsorption column was 4800ml/5200m/5000ml, and the volume of plasma replaced was

800ml/1000ml/1000ml. the treatment time was 172min/185min/196min, and the treatment process was smooth, and the patient's vital signs were stable.

Results:
The total bilirubin before the first plasma exchange combined with bilirubin adsorption treatment was 735umol/L, and the total bilirubin after treatment decreased to 278umol/L, with a clearance rate of 62%; the total bilirubin before the second plasma exchange combined with bilirubin adsorption treatment was 558umol/L, and the bilirubin detected at 10 hours after the treatment was 381umol/L, with a clearance rate of 31. 7% (the rebound phenomenon needs to be considered); the third plasma exchange combined with bilirubin adsorption treatment was 550.5umol/L before treatment, and it was 60% after treatment. rebound phenomenon); the total bilirubin before the third plasma exchange combined with bilirubin adsorption treatment was 550.5 umol/L, and after treatment it was 217.8 umol/L, with a clearance rate of 60.4%.

Conclusion:
In patients suffering from severe hepatic GVHD following allogeneic haematopoietic stem cell transplantation, particularly those exhibiting significantly elevated bilirubin levels, the combination of plasma exchange with bilirubin adsorption therapy has been shown to effectively reduce bilirubin indexes. This approach has been demonstrated to prevent the onset of hepatic encephalopathy, bilirubin encephalopathy and other serious complications, thereby providing patients with a valuable therapeutic window to ensure the subsequent administration of treatments is conducted in a timely manner, thus enhancing the overall therapeutic efficacy.

Poster Presentation

Graft-versus-host Disease

Skin Chronic Graft-versus-host Disease following Autologous Hematopoietic Stem cell Transplantation for Primary Central Nervous System Lymphoma

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Background: Graft versus host disease (GVHD) is a common complication after allogeneic hematopoietic stem cell transplantation (HSCT). Chronic GVHD typically occurs after 3 months HSCT and can potentially be associated with significant morbidity and mortality. This autoimmune-like mechanism characterized by impaired immune tolerance, due to alloreactivity of donor-derived T and B cells can present in several organs such as skin, lung and gastrointestinal system. However, GVHD is rarely seen in autologous HSCT (AHSCT) since there is absence of alloreactivity. Here we report a case of autologous GVHD (auto-GVHD) involving the skin after AHSCT for primary central nervous system lymphoma (PCNL).

Case report: a 62 year-old man diagnosed with PCNL was admitted for AHSCT after 6 cycles of chemotherapy of temozolamide, methotrexate and rituximab. He achieved complete remission after six cycles of the chemotherapy. On day 92 post-transplant, he developed generalized maculopapular rashes involving abdomen, back, face and upper and lower limbs, approximately 75% of his total body surface. Skin biopsy showed inflammatory infiltrate at the dermal surface, lichenoid pattern of inflammation with perivascular neutrophilic and lymphocytic cells infiltration, consistent with chronic skin GVHD. Periodic acid Schiff (PAS), Gomori Methenamine Silver (GMS) and Ziehl-Neelson staining were all negative. He was treated with prednisolone 0.5mg/ kg/day, with dose of 30mg daily. He responded well with prednisolone and achieved complete response after 10 weeks of prednisolone without any recurrence.

Discussion: GVHD in AHSCT is an uncommon complication from AHSCT. The pathophysiology of auto GVHD is uncertain although there were theories related to immune dysregulation, loss of self-tolerance, thymic damage and molecular mimicry which may related to prior exposure of drugs such as bortezomib and alemtuzumab. There were only few cases reported and majority responded to corticosteroid. In this patient, the prior exposure to temozolamide may play a role since mechanism of action involves depleting T-regulatory (T-reg) cells, contribute to loss of self-tolerance and autoimmune-like reactions.

Conclusion: Although GVHD is rare in AHSCT, patients present with typical rash should alert physician to include auto-GVHD as one of the differential diagnoses and biopsy should be performed in order appropriate treatment can be implemented.

Poster Presentation

Graft-versus-host Disease

Telegram-based Gaming Platform for Teaching Chronic GVHD Staging and Treatment

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Introduction: Russia's extensive territory and uneven distribution of specialized medical centers present challenges for healthcare providers seeking advanced training in a field of allo-HSCT and cGVHD espessially. Remote education facilitates access to high-quality educational content, enabling specialists in remote or underserved regions to stay current with the latest advances, clinical protocols, and evidence-based practices without extensive travel.
Gamification refers to the integration of game design elements, principles, and mechanics into educational contexts to enhance learner engagement, motivation, and outcomes. It transforms traditional learning experiences into interactive, immersive, and enjoyable processes.

Aim: To develop an online gaming platform for teaching doctors the principles of staging and treatment of chronic GVHD.

Results: We developed Telegram-based educational game designed to enhance knowledge through interactive engagement directly within the messenger interface. Users interact with a game get knowledge about NIH staging and treatment approaches for chronic GVHD. Players answer questions and receive instant feedback. Additional information about drugs and their effectivness give unobtrusive learning in a field of cGVHD. Leaderboards encourage friendly competition among users, fostering motivation and repeated participation. We will also collect information about the chosen approaches before and after learning in the game.
The game is available on gvhd.ru

Poster Presentation

Graft-versus-host Disease

The Efficacy and Safety of a Reduced Dose Post Transplantation Cyclophosphamide (35mg/kg) as GVHD Prophylaxis

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INTRODUCTION:
Post-transplantation cyclophosphamide (PTCy) has become a widely used approach for patients with leukemia undergoing allogeneic stem cell transplantation. PTCy at 35 mg/kg is preferred over higher doses like 50 mg/kg because it achieves a balance between toxicity reduction and faster engraftment. PTCy is used as GVHD prophylaxis, reducing the risk of relapse and lowering the incidence of GVHD following hematopoietic stem cell transplantation. Evidence indicates that a reduced dose of 35 mg/kg may offer comparable prevention of GVHD without compromising event-free survival or increasing non-relapse mortality.

AIM:
To study the efficacy and safety of a reduced dose (35 mg/kg) Post transplant cyclophosphamide

METHODS:
We retrospectively analyzed outcomes of patients who received PTCy 35mg between 2023–2024, with a minimum follow-up of 3 months.

RESULTS:
A total of 48 patients were included in the study. Acute myeloid leukemia (AML) was the underlying disease in most cases — 22 patients (45%). Other diagnoses included acute lymphoblastic leukemia (ALL) in 17 (35%), myelodysplastic syndrome (MDS) in 2 (4.16%), Angioimmunoblastic T-cell lymphoma (AITL) in 1 (2%), multiple myeloma in 1 (2%), peripheral T-cell lymphoma (PTCL) in 1 (2%), and chronic myeloid leukemia (CML) in 2 patients (4. 16%).Peripheral blood was the stem cell source in 100% of cases. RIC was administered in 25 patients (52.3%), and MAC in 23 patients (47.9%). Donors were Haplo identical in 34 patients (70.3%), MSD in 9 (18.7%), and MUD in 5 (10.41%). Median Age of patients (range 11-64 years) was 37.5 years. Patients underwent transplantation with a median CD34+ cell dose of 6.5 × 10^6 cells/kg (Range 3.19-24x10^6cells/kg). Other complication included

hemorrhagic cystitis in 4 Patients(8.3%),BK virus infection in 5(10%) and bacterial infection in 22(45%) Median time to neutrophil engraftment was 15 days (Range 12-25), and median time to platelet engraftment was 16 days (Range 10-36). Relapse occurred in 6 patients (12.5%). Event-free survival was observed in 31 patients (64.58%), overall survival in 36 patients (75%), and non-relapse mortality (NRM) in 11 patients (22.9%). GVHD was seen in 20 patients (41%), with acute GVHD occurring in 14 patients (29%) and chronic GVHD in 10 patients (20%). Full donor chimerism was observed in 43 patients (88%) at Day 30 and in 38 patients (79%) at Day 100.

CONCLUSION:

Our retrospective analysis suggests that PTCY(35 mg/kg) is an effective and safe alternative to the standard 50 mg/kg dose for GVHD prophylaxis in allogeneic stem cell transplantation. The reduced dose was associated with favorable outcomes, including high rates of full donor chimerism, acceptable engraftment times, manageable transplant-related complications, and promising event-free and overall survival rates. Importantly, relapse rates and non-relapse mortality remained within acceptable limits, supporting the potential use of the reduced dose as a viable strategy to minimize toxicity without compromising efficacy.

Poster Presentation

Graft-versus-host Disease

Incidence, Severity, and Outcomes of Acute and Chronic Graft-versus-Host Disease Following Peripheral Blood Allogeneic Stem Cell Transplantation in Myeloid Malignancies: A Single-Center Study from a Tertiary Care Hospital in Southern India

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Aims:
Despite advances in prophylaxis and therapy, graft-versus-host disease (GVHD) remains a significant complication affecting patient outcomes, especially as more allogeneic stem cell transplants (SCT) are performed using peripheral blood stem cells and across the HLA barrier. Here, we present the incidence, severity, and outcomes of acute and chronic GVHD in patients undergoing matched sibling donor (MSD) and haploidentical SCT at Aster MIMS Hospital, Kozhikode, Kerala, India.

Methods:
Hospital data of 22 patients who underwent allogeneic SCT (MSD or haploidentical) for acute myeloid leukemia (AML), chronic myeloid leukemia (CML) in blast crisis, myelodysplastic syndrome (MDS), and juvenile myelomonocytic leukemia (JMML) between January 2022 and December 2024 were retrospectively analyzed. Two patients were excluded—one due to primary graft failure and the other due to early post-engraftment mortality from relapsed AML. Acute GVHD was graded using the Mount Sinai Acute GVHD International Consortium (MAGIC) criteria, and chronic GVHD was graded according to the National Institutes of Health Consensus Conference criteria.

Results:
Twenty eligible patients with myeloid malignancies were included in the analysis. Nine patients (45%) were adults. The median age was 16.5 years (range 3–54 years), and nine patients (45%) were female. Twelve patients (60%) had AML. Haploidentical SCT was performed in 10 patients (50%). Post-transplant cyclophosphamide (PTCy)-based GVHD prophylaxis was used in 12 patients (60%). The median CD34+ cell count was 7.4 million cells/kg (range 5.8–12.5 million

cells/kg). For haploidentical transplants, the median CD3+ cell count was 250 × 10^6 cells/kg (range 100–420 × 10^6 cells/kg). The median time to neutrophil engraftment was 12.5 days (range 10–18 days). Grade 3 mucositis was observed in eight patients (40%).

Acute GVHD (all grades) was observed in 12 patients (60%), of whom four (33%) responded to topical steroids for limited skin involvement without the need for systemic steroids. Chronic GVHD (all grades) was observed in seven patients (35%). Grade 2–4 acute GVHD occurred in two patients (10%), while moderate or severe chronic GVHD occurred in three patients (15%). Systemic corticosteroid therapy was effective in five of eight patients (62.5%) with acute GVHD and in three of seven (42%) with chronic GVHD.

Ruxolitinib was used as second-line therapy in steroid-refractory GVHD. A complete response was observed in one of three patients (33%) with acute GVHD and in two of four patients (50%) with chronic GVHD. The overall survival and progression-free survival were 75% and 65%, respectively, at the time of analysis. Of the five deaths, three were due to disease relapse, one due to disseminated fungal infection, and one due to refractory chronic lung GVHD despite treatment with ruxolitinib, etanercept, and extracorporeal photopheresis.

Conclusions:
This study highlights the significant incidence of acute GVHD in patients undergoing allogeneic SCT for myeloid malignancies at our center over the past three years. The data also suggest that ruxolitinib shows a more pronounced response in chronic GVHD compared to acute GVHD.

Poster Presentation

Graft-versus-host Disease

Ex-vivo TCRαβ and CD45RA-Depleted Grafts in Mismatched Unrelated Donor Hematopoietic Stem Cell Transplantation: A Case Series from Singapore

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Aim/Background:
Mismatched unrelated donors (MMUD) are a viable donor source for patients lacking HLA-matched donors. However, the optimal graft-versus-host disease (GVHD) prophylaxis strategy in this setting remains undefined. Our centre has previously demonstrated that ex-vivo depletion of $TCR\alpha\beta^+$ and $CD45RA^+$ naïve T cells with add-back of $CD45RO^+$ memory T cells yield favourable outcomes in haploidentical transplants (*Koh LP et al, Blood (2022) 140 :4707–4708*). Here, we report a case series of MMUD transplants using the same protocol to assess feasibility, engraftment, and post-transplant outcomes.

Methods:
We retrospectively analysed three adult patients with hematologic malignancies who underwent MMUD allogeneic hematopoietic stem cell transplantation in our centre. They received reduced-intensity conditioning regime comprised of fludarabine, thiotepa, melphalan and total body irradiation (2 Gy). All patients received short-course tacrolimus for 30 days as GVHD prophylaxis. A minimum $CD34^+$ cell dose of 5×10^6/kg was requested. Key endpoints included neutrophil/platelet engraftment, acute and chronic GVHD, relapse, non-relapse mortality (NRM), and infectious complications.

Results:
Patient 1: 57-year-old female with relapsed intermediate-risk MDS (trisomy 8, U2AF1 mutation), previously treated with syngeneic transplant in 2011. She relapsed and developed presumed MDS-related autoimmune colitis. A single cycle of azacitidine resulted in severe pancytopenia. She underwent MMUD transplant (HLA-B allele mismatch) in February 2023, receiving $TCR\alpha\beta$-depleted $CD34^+$ and $CD45RO^+$ doses of 7.45×10^6/kg and 5.99×10^6/kg, respectively. Neutrophil and platelet engraftment occurred on Days +12 and +14, with full donor chimerism by Day +14. She developed Grade II acute GVHD (skin), which responded to steroids.

Bone marrow post-transplant confirmed the remission status. She remains in remission at 2 years without serious infections.

Patient 2: 60-year-old male with relapsed AML (ASXL and RUNX1 mutations) in second complete remission. He underwent MMUD transplant (HLA-A allele mismatch) in March 2024, receiving TCRαβ-depleted $CD34^+$ and $CD45RO^+$ doses of 5.99×10^6/kg and 2.15×10^6/kg, respectively. Neutrophil and platelet engraftment occurred on Days +18 and +16, with full donor chimerism by D+36. He developed Grade I acute GVHD. Day +90 marrow showed MRD at 0.7% by flow. Given high-risk features, he received donor lymphocytes infusion (DLI) twice. He remains in remission at 1-year post-transplant without serious infection.

Patient 3: 55-year-old female with primary refractory AML (trisomy 21, WT1+) and achieved pre-transplant MRD of 1.9%. She underwent MMUD transplant (HLA-DRB1 allele mismatch) in August 2024 with a TCRαβ-depleted graft ($CD34^+$ 8.91×10^6/kg; $CD45RO^+$ 2.23×10^6/kg), following donor specific antibodies desensitization with plasma exchange and rituximab. Neutrophil and platelet engraftment occurred on Days +12 and +15, with full donor chimerism by Day +27. She developed Grade I acute GVHD. Day +60 marrow showed MRD 0.89% by flow. She received DLI once, resulting in MRD reduction to 0.13%. She remains in remission at 8-months post transplant.

Conclusion:

This early experience suggests that MMUD transplantation using ex-vivo TCRαβ and CD45RA depletion with memory T-cell add-back is a feasible and safe approach. It resulted in reliable engraftment, low-grade acute GVHD, with no early NRM, relapse and chronic GVHD. These encouraging findings support further evaluation of this strategy in larger prospective studies.

Poster Presentation

Graft-versus-host Disease

Neutrophil IDO1-AhR Axis Regulates T Cell Proliferation and GVHD Risk After Haploidentical Stem Cell Transplantation

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According our preview study, we mentioned that post-transplant neutrophils inhibited of T cell proliferation. Clinical outcomes indicated that higher neutrophil-mediated T cell suppression is associated with a reduced GvHD incidence in our study cohort. Using RNA-seq to discover molecular mechanism, IFNγ signature is the most significant gene set in post-transplant neutrophils. We found the expression of indoleamine 2,3-dioxygenase 1 (IDO1), the IFNγ-induced gene, is higher in post-transplant neutrophils. Most studies reveal tolerance dendritic cells, suppressive macrophage, and tumor cells release IDO1 to consume tryptophan then inhibit T cell proliferation. To investigate whether post-transplant neutrophils modulate T cell proliferation through IDO1, we collect more patient specimens to verify. Either gene expression or intracellular protein expression of IDO1 from patients received haploidentical transplant are higher than healthy donor, patients received auto-transplant patients, and patients received matched related donor (MRD) transplant. Comparing with immature $CD10^{+}CD16^{-}$ neutrophil, mature $CD10^{+}CD16^{+}$ neutrophil from patient has higher IDO1 and aryl hydrocarbon (AhR) receptor expression. Meanwhile, $CD4^{+}$ or $CD8^{+}$ T cells from patient with mature $CD10^{+}CD16^{+}$ neutrophil has lower Ki67 expression, a proliferation marker. AhR is a ligand-activated transcription factor which senses tryptophan metabolites to activate IDO1-downstream gene expression. These data indicate mature $CD10^{+}CD16^{+}$ neutrophil from post-transplant neutrophil has higher IDO1 and AhR expression may reduce T cell proliferation.

AhR manipulates regulator T (Treg) cell differentiation, therefore, we investigate whether neutrophils suppression T cell proliferation through upregulating Treg cell formation. However, we found the percentage of Treg cells in peripheral blood from suppressive neutrophil cohort or non-suppressive neutrophil cohort is similar. Using IFNγ pre-treat neutrophil from healthy donor to inhibit T cell proliferation, we found this neutrophil promote AhR^{+} T cell proliferation instead of Treg cells, and using IDO inhibitors or AhR antagonist could diminish AhR^{+} T cell proliferation.

Notably, in non-suppressive neutrophil cohort, pre-treating IFNγ neutrophil also could promote AhR^+ T cell proliferation but fail to inhibit total T cell proliferation.

To decipher the T cell phenotype from post-transplant patient peripheral blood, we found the percentage of $CD8^+CD28^+CD57^+$ is higher in non-suppressive neutrophil cohort. $CD8^+CD28^+CD57^+$ T cells have higher co-stimulatory molecules, pro-inflammatory cytokines, and chemokine receptors expression. While antigens explosion, naïve T cell ($CD28^+CD57^-$) will differentiation into active $CD8^+CD28^+CD57^+$ T cells then lost CD28 expression subsequently. In fact, the percentage of $CD8^+CD28^+CD57^-$ T cells gradually reduce during immunologic reconstitution after haploidentical transplant. Taken together, we speculate autoantigens explosion stimulate IFNγ signaling in post-transplant neutrophil, especially in the haploidentical transplant cohort. IFNγ trigger IDO1 expression thereby promoting AhR^+ T cell proliferation. Nerveless, suppressive neutrophil cohort has less $CD8^+CD28^+CD57^+$ T cells in peripheral blood, whether IDO1-AhR signaling interfere $CD28^+CD57^+$ T cells formation need to be investigate. A few observations pointed the neutrophil function during immunologic reconstitution in transplant patients, our study provide a novel perspective to decipher the interaction between neutrophil and T cells then predict the GVHD development.

Poster Presentation

Infectious and Non-infectious complications

Bioequivalent Generic Letermovir Prophylaxis in CMV-Seropositive Allogeneic HSCT Recipients: A Retrospective Multi-Centre Analysis in Indian patients

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Aims:
To evaluate the real-world effectiveness and safety of bioequivalent generic Letermovir (Anvimo) for cytomegalovirus (CMV) prophylaxis in CMV-seropositive patients undergoing allogeneic hematopoietic stem cell transplantation (Allo-HSCT) across multiple transplant centers in India.

Methods:
This retrospective, observational, multi-centre study analysed data from 10 patients who underwent Allo-HSCT and received Bioequivalent Generic Letermovir prophylaxis. Data were sourced from Hospital database. Variables assessed included patient demographics, Clinical characteristics, Letermovir prophylaxis details, CMV reactivation, Safety profile and discontinuation events.

Results:
Patient Demographics: The cohort included 7 male and 3 female patients, of which 6 received Transplant for Acute Myeloid Leukaemia, 2 for classical Hodgkin lymphoma, 1 for Wiskott-Aldrich syndrome and 1 for Thalassemia Major. 7 Haploidentical, 2 Matched Unrelated and 1 Matched Related Transplant patients received 5 RIC, 4 MAC and 1 NMA conditioning. PtCy, Tacrolimus and Cyclosporin were the most used GVHD prophylaxis, and 2 patients developed, Acute post-transplant GVHD. 1 patient developed Mild Gastro-Intestinal Adverse event.
Generic Bioequivalent Letermovir Prophylaxis was initiated at a median of 1 (0-4) day post-transplant and continued for a median duration of 100 (47-200) days. Clinically significant CMV infection occurred in No patient during Letermovir prophylaxis period, 1 out of 10 of patients developed CMV infection, post Letermovir prophylaxis was discontinued.

Conclusion:
This retrospective multi-center analysis provides preliminary real-world evidence

supporting the effectiveness and safety of Bioequivalent Generic Letermovir for CMV prophylaxis in CMV-seropositive allogeneic HSCT recipients in India.

Poster Presentation

Infectious and Non-infectious complications

Convalescent Plasma Therapy for COVID-19 Prophylaxis in Adults Early Post-Hematopoietic Stem Cell Transplantation: One-Year Outcomes from a Randomized Controlled Trial

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Objective: The study aimed to evaluate the efficacy and safety of COVID-19 convalescent plasma (CCP) in preventing COVID-19 among adult hematopoietic stem cell transplantation (HSCT) recipients during the early post-transplant period.

Design: A single-center, randomized controlled trial conducted between June 2023 and February 2024.

Setting: Hematopoietic Stem Cell Transplantation Center of the Institute of Hematology & Blood Diseases Hospital.

Participants: Out of 313 patients screened, 72 were randomized into either the CCP group or standard treatment group (36 patients each).

Intervention: Participants were randomly assigned in a 1:1 ratio to either the CCP group or the standard treatment group. The CCP group received 200 ml of COVID-19 CCP at +14 days, +28 days, +2 months, and +3 months post-transplantation, in addition to the standard oral ursodeoxycholic acid. The standard treatment group received ursodeoxycholic acid routinely.

Main Outcome Measures: The primary outcome was the incidence of COVID-19 within 28 days after the last CCP infusion. Secondary outcomes included the incidence of severe COVID-19 within 28 days after the last CCP infusion, the survival rate within 30 days of COVID-19 infection, overall survival at one-year post-transplantation, and adverse reactions to CCP infusion.

Results: A total of 72 participants tested negative for SARS-CoV-2 at baseline and were included in the Intention-to-Treat (ITT) population. Of these, 62 patients

(86.1% of the ITT population) were included in the per-protocol (PP) analysis, and no patients were lost to follow-up during the study. The cumulative incidence of COVID-19 infection within 28 days after the last CCP infusion was 26.9% (95% CI, 7.7–42.1%) in the CCP group and 22.9% (95% CI, 7.6–35.6%) in the standard treatment group, with no significant difference (p = 0.786). Severe infections were uncommon, occurring in 3.9% (95% CI, 0–10.1%) and 2.8% (95% CI, 0–8.0%) of patients in the CCP and standard treatment groups, respectively (p = 0.806). In terms of one-year overall survival (OS), rates were 79.2% (95% CI, 64.4–97.4%) in the CCP group and 91.7% (95% CI, 83.1–100%) in the standard treatment group (p = 0.182).The survival rate within 30 days of COVID-19 infection was 100% in both groups. Among the 36 patients who received CCP, three experienced mild adverse events (fever or rash), all of which resolved quickly with supportive care, and no severe adverse reactions were observed.

Conclusion: CCP therapy did not reduce the incidence or severity of COVID-19. Moreover, the one-year overall survival was significantly lower in the CCP group compared to the standard treatment group. These findings do not support the use of CCP therapy as a preventive measure for COVID-19 in patients post-hematopoietic stem cell transplantation.

Trial registration: ClinicalTrials.gov NCT05904067.

Poster Presentation

Infectious and Non-infectious Complications

The dosage matters to achieve the sufficient rise of immunoglobulin levels using immunoglobulin replacement therapy in allogeneic transplant recipients

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Aims:
Secondary hypogammaglobulinemia (SHG) is a common complication after hematopoietic cell transplantation (HCT), particularly in recipients of allogeneic HCT. SHG significantly increases the risk of recurrent and severe infections, negatively impacting post-transplant outcomes. Immunoglobulin replacement therapy (IgRT) is widely used to mitigate this risk, but the optimal dosing strategy for IgRT after HCT remains controversial. While sufficient IgG trough levels are considered important, the relationship between ScIgRT dosage and IgG response has not been well characterized in this population. This study aimed to investigate the impact of ScIgRT dosage on changes in serum IgG levels in patients with SHG following HCT.

Methods:
This retrospective study was conducted at a single center and included patients who received subcutaneous IgRT (ScIgRT) at least 60 days after autologous or allogeneic HCT between 2017 and 2023. Inclusion criteria required measurement of serum IgG levels within 1 week before and 1 month (±1 week) after the initiation of ScIgRT. Patients who received intravenous IgRT within 1 month prior to ScIgRT initiation or had active relapse or progression of their hematologic malignancy were excluded. Clinical characteristics were collected, and changes in IgG levels were analyzed. Patients were stratified by ScIgRT dosage (<24 g/month vs. ≥24 g/month; and <0.4 g/kg/month vs. ≥0.4 g/kg/month). Statistical analyses were performed using the Mann–Whitney U test and correlation analyses were conducted using Pearson's correlation coefficient. A p-value <0.05 was considered statistically significant.

Results:
A total of 81 patients met the inclusion criteria. The median age at HCT was 57 years (range, 19–75 years), and 73 patients (90.1%) underwent allogeneic HCT. The

median time from HCT to ScIgRT initiation was 124 days. The median serum IgG level before ScIgRT was 669 mg/dL, which significantly increased to 780 mg/dL one month after ScIgRT initiation ($P < 0.01$). There was a significant positive correlation between the monthly ScIgRT dosage and the increase in IgG levels ($r=0.51$, $P<0.01$). Patients who received ≥24 g/month of ScIgRT showed a greater median increase in IgG levels compared to those who received <24 g/month (163 mg/dL vs. 59 mg/dL, $P<0.01$). Similarly, patients who received ≥0.4 g/kg/month demonstrated a significantly higher median increase compared to those who received <0.4 g/kg/month (222 mg/dL vs. 59 mg/dL, $P<0.01$).

Conclusions:

Our study demonstrated that higher dosages of ScIgRT were significantly associated with greater increases in serum IgG levels in patients with SHG after HCT. These results suggest that optimizing ScIgRT dosing is essential to achieve sufficient IgG levels, which may potentially reduce the risk of infections. Prospective studies with long-term monitoring of IgG levels and infection rates are needed to validate the clinical benefit of maintaining higher IgG trough levels in this patient population.

Poster Presentation

Infectious and Non-infectious Complications

Analysis of the Safety and Efficacy of Luspatercept Use Following Allogeneic Hematopoietic Stem Cell Transplantation in Patients with Hematologic Malignancies

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Backgroud: Luspatercept is a specific activin receptor fusion protein that reduces SMAD2 and SMAD3 signaling by binding specific transforming growth factor β (TGF-β) superfamily ligands, thereby allowing erythrocyte maturation through late-stage erythroblast differentiation [3]. Based on the clinical trial data, luspatercept is the first and only FDA-approved red blood cell maturation agent to help patients reduce the red blood cell infusion burden.

Aim: To evaluate the safety and efficacy of luspatercept in promoting erythroid engraftment post-allo-HSCT in patients with hematologic malignancies.

Methods: From December 2021 to January 2025, we retrospectively analyzed 132 allo-HSCT patients: 93 received Luspatercept (1 mg/kg initial dose; median 1 dose, range 1-2) and 39 served as controls. Median follow-up was 16.4 months (95%CI 12.4-18.1).

Results: A total of 132 patients with hematologic malignant diseases who underwent allo-HSCT, This cohort comprised 77 males (58.3%) and55 females (41.7%), with 32 children (<18 years) and 100 adults (≥18 years). Diagnoses included AML (n=51, 38.6%), B-ALL (n=27 , 20.5%), NKTCL (n=25 , 18.9%), T-ALL (n=10, 7.6%), T-LBL (n=8, 6.1%), MDS (n=4 , 3.0%) and others (n=7, 5.3%). Fifty-one (38.6%) cases underwent the second transplants.80 patients achieved CR (60.6%) before transplantation. Myeloablative conditioning regimens included TBI/Flu (49.2%, n=65), BU/Flu (50.8%, n=67) .Donor types included haploidentical (63.6%, n=84), unrelated (34.1%, n=45) and identical siblings (2.3%, n=3). Two people had received other ESAs treatment. All patients were in CR, and CD3 and CD34 chimeric rates were complete donor type.
Luspatercept was initiated at day +7 post-allo-HSCT. Key hematologic findings: Overall cohort: Significant hemoglobin elevation in the treatment group at 1 week post-administration (108±12 g/L vs 92±10 g/L in controls, p=0.009). Adult

subgroup: Sustained hemoglobin improvement at both 1 week (p=0.012) and 2 weeks (p=0.07).Pediatric subgroup: Significant hemoglobin increase at 1 week (105±15 g/L vs 89±12 g/L in controls, p=0.009). There was no significant difference in the hemoglobin concentration in the two groups after 2 weeks. The median time from the initiation of Luspatercept treatment to the erythrocyte response were 7 (4-14)days. We also observed that the Luspatercept group had significantly lower IL-10, IL12p70 and IL-8 two week after medication use than the control group (IL-10: p=0.034; IL12p70: p=0.023; IL-8: p=0.006). The probability of aGVHD occurring after transplant in the Luspatercept group was slightly higher than that in the control group, with no significant difference (39.8% vs 35.9%, p=0.824). The luspatercept group showed a significantly reduced incidence of post-transplant viremia versus controls (53.8% vs 76.3%; p=0.018). Luspatercept-related adverse events occurred in 15.3% of patients (fatigue, palpitations, limb edema), all grade 1. No grade ≥2 toxicities were observed.

Conclusion: Luspatercept demonstrates safety and efficacy in post-allo-HSCT patients, rapidly improving hemoglobin (within 1 week), reducing cytokines and aGVHD incidence, without increasing viremia risk.

Poster Presentation

Infectious and Non-infectious complications

Acute Appendicitis as a Harbinger of Relapsed Aplastic Anemia Following Allogeneic Hematopoietic Stem Cell Transplantation

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BACKGROUND:
Relapsed severe aplastic anemia after allogeneic hematopoietic stem cell transplantation (allo-HSCT) typically presents as gradual cytopenias, often detected through routine blood counts and donor chimerism. While infections are common post-transplant, acute appendicitis as an early sign of graft failure is exceedingly rare. The appendix, involved in mucosal immune surveillance and T-cell priming, may serve as early immunologic sentinel. We report a rare case in which appendicitis was the first manifestation of marrow relapse, suggesting a link between localized mucosal immune dysregulation and graft dysfunction.

CASE:
A 21-year-old female with severe aplastic anemia underwent allo-HSCT from a 7/8 HLA-matched sibling using rabbit ATG and cyclophosphamide as conditioning. She engrafted by Day +18 with full donor chimerism by Day +28. Cyclosporine A was discontinued on Day +103. On Day +161, she developed high- grade fever and severe abdominal pain. CT scan confirmed acute appendicitis. She underwent open appendectomy. While admitted, new-onset pancytopenia was appreciated but attributed to infection. Post- discharge, cytopenias worsened. She was re-admitted for fever and diarrhea, tested positive for Clostridium difficile, and started on oral vancomycin. Bone marrow biopsy revealed hypocellularity, confirming relapsed aplastic anemia. Eltrombopag and cyclosporine A were restarted, and second allo-HSCT planned. On third week of admission, she developed hemoptysis, and desaturation. Chest CT showed right pericardiac consolidation. CT-guided biopsy confirmed aspergillosis. Despite aggressive antifungal therapy, she deteriorated and succumbed to acute respiratory failure from invasive pulmonary aspergillosis.

CONCLUSION:
This case highlights acute appendicitis post-HSCT as potential sentinel event signaling immune dysregulation and impending marrow failure. It poses critical insight whether localized infections in lymphoid-rich organs like appendix may

precede hematologic decline, warranting earlier bone marrow evaluation and heightened infectious surveillance. Clinicians should maintain high index of suspicion and consider prompt marrow and chimerism assessment when unexpected focal infections emerge—even in the absence of overt cytopenias.

Poster Presentation

Infectious and Non-infectious complications

Clinical analysis of 4 patients with PT-AIHA

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OBJECTIVE: To report 4 cases of PT-AIHA (post-transplantation autoimmune hemolytic anemia) that occurred in our center in recent years, with the aim of increasing clinical attention and management of this rare transplant-related complication.

METHODS: 4 patients were enrolled, all of whom were patients with hematopoietic lymphoid malignancies and had received allo-HSCT (allogeneic hematopoietic stem cell transplant), 3 males and 1 female, and in terms of donor, 1 was MSD (matched sibling donor), and 3 were HID (haploidentical donor). Anemia occurred from 10 months post-transplantation to 22 months post-transplantation, and all showed severe-very severe anemia (Hb 25-35 g/L) at the time of occurrence, and the primary tumors were in CR status (complete remission) at the time of anemia. The diagnosis of PT-AIHA was clarified by means of laboratory tests such as elevated reticulocytes, elevated bilirubin, elevated lactate dehydrogenase, detection of erythrocyte fragments, and a positive Coombs test. PRCA (pure red cell aplasia) was screened by microvirus B19, TMA (thrombotic microangiopathy) by soluble CD25, and graft rejection or primary tumor recurrence by post-transplantation chimerism test.

RESULTS: For treatment, after 1-1.2 mg/kg.d of MP (methylprednisolone)± high-dose IVIG (intravenous immunoglobulin) therapy, and the addition of calmodulin inhibitors in 2 patients with unsatisfactory initial results, good results were achieved, with a minimum of only 4 days and a maximum of 31 days to get out of the moderate-to- severe anemia (Hb>90 g/L). Median follow-up was 19 months, with no recurrence of hemolysis.

Summary: In these 4 cases of PT-AIHA observed in our center, the events occurred in the middle to late post-transplantation period, and the events were not related to the donor-recipient blood group compatibility. The diagnostic criteria refer to primary AIHA, which needs to be differentiated from post-transplant PRCA, TMA, graft rejection and primary tumor recurrence. Treatment was based on primary AIHA with high- dose MP± IVIG and the addition of calmodulin inhibitors in cases where initial efficacy was unsatisfactory, with an overall favorable outcome and no

adverse prognosis noted. Rituximab was used in only 1 patient, suggesting to some extent that rituximab is an option rather than a necessity in the treatment of PT-AIHA.

Poster Presentation

Infectious and Non-infectious complications

Pure red cell aplasia –Post Major ABO incompatible allogenic stem cell transplantation role of Ibrutinib

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Pure red cell aplasia (PRCA) is a well recognized complication of Major ABO incompatible allogenic stem cell transplantation. PRCA has major morbidity in the terms of transfusion and other approaches. Various treatments have been tried with heterogeneous response. Ibrutinib – tyrosine kinase inhibitor used approved for CLL and MCL, inhibit the Btk which prevents the downstream activation of BCR pathway there by subsequently block cell growth, proliferation and survival of B cell. In PRCA Ibrutinib helps by blocking the B cell pathway there by plasma cell and antibody production. This study is the new explored clinical indication for Ibrutinib.

Introduction:

Pure red cell aplasia (PRCA) can occur following allogenic stem cell transplantation if donor and recipient ABO blood groups are mismatched, with the recipient having isoagglutinins against the donor blood group(1). Plasma cells that survive despite conditioning produces anti-ABO isoagglutinins which target donor erythroid precursors in the bone marrow thereby cases PRCA.PRCA occurred in about 7% of Major ABO mismatched HCTs it can present either as PRCA or pancytopenia (2). Therapeutic options includes steroids, discontinuation of immunosuppression, Bortezomib, rituximab, daratumumab and donor lymphocyte infusion, all having a variable success. Ibrutinib – small molecule tyrosine kinase inhibitor works against BTK receptor on B cells there by reduces the production of plasma cells and isoagglutinins, which makes it probably potential option in PRCA in HCTs.
Here we are reporting two such cases successfully managed with Ibrutinib, patient become transfusion independent and their blood group changed in 4weeks duration.

Methods:

This is the retrospective study collected data from the clinical registry, PRCA was confirmed by bone marrow aspiration and biopsy. Those who were requiring transfusion more than 90days following allogenic stem cell transplantation, with no response with previously described agents like EPO agonist, Rituximab, Bortezomib trail drug Ibrutinib was introduced and their hemoglobin, Anti A and B titers, blood grouping both forward and reverse grouping was done at intervals.

Results and discussion:

In this case series we are reporting 7 case series, PRCA post allogenic HCT successfully treated with Ibrutinib. After obtaining institute ethical committee permission 7 patient were enrolled for drug. The dose of Ibrutinib was initiated at 140mg/day depending on response dose was adjusted and maximum dose allowed was 420mg/day. All 7 patient had a response within 3weeks and they become transfusion independent and response also documented with Anti A and Anti B titer, no major toxicities noticed and no breakthrough CMV and EBV infections in the series. PRCA is a well known effect of ABO mismatched allogenic HCT, documented in about 7% of ABOi HCTs(3). ABO mismatches transplants can produces either PRCA alone or PRCA with Pancytopenia(4). Various agents have been used in the literature like pharmacological EPO agonist, Rituximab, Bortezomib and Daratumumab, Non-Pharmacological approach like Tapering and stopping of immunosuppression, DLI and stem cell boosting(5). All these agents having variable success rate between 40-60%. In literature majority of patient were blood group O, even in our series both patient were blood group O(2). However, Ibrutning though it is not yet approved for this indication there is one single center publication of 5 patient data(6). Our series is the second in literature and largest for using the Ibrutinib for PRCA post HCT. The countries like India Ibrutinib is much economical than other pharmacological agents in view generic availability so it also a cost effective option. Ibrutninb was well tolerated at small dose 140mg per day, no major adverse effects except one patient developed Periungual granuloma which was treated with topical Boric acid(7). Seven patient become transfusion independent with in 4weeks and blood group was changed with in 6weeks, similar pattern was demonstrated in the Arslan et all publication(5). Our experience shows that Ibrutinib could give safe and effective therapeutic treatment option for Refractory PRCA post HCT. Prospective trails required to assess if early introduction would reduces the morbidity of transfusion, cost saving and GVHD complication if co-exist.

Conclusions:

Pure Red cell aplasia post allogenic stem cell transplant one of the major morbidity, various modalities have been tried with heterogeneous success. Ibrutinib is a new armamentarium, which has shown near 100% response in the limited published data. It needs a large trial to confirm the findings.

Poster Presentation

Infectious and Non-infectious complications

Oral Mucositis Following Stem Cell Transplant – A Study Of 20 Consecutive Patients From A Single Centre

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Aims:
To describe the risk factors, severity and management strategies used for oral mucositis (OM) following various conditioning regimen in autologous and allogenic stem cell transplants performed at our centre.

Methods:
Patients who underwent stem cell transplants at our centre over a 6-month period were clinically evaluated for the development of oral mucositis and graded for severity based on World Health Organization toxicity scale as follows:

Grade 0- No OM

Grade 1 - Oral soreness and erythema

Grade 2- grade 1 + ulcers

Grade 3 – extensive ulcers- only liquid diet tolerated

Grade 4 – oral alimentation not possible

Data collected included demographics, transplant details, conditioning regimen, therapeutic interventions and time to onset and resolution of mucositis. Study end point was the resolution of OM.

Results:
20 consecutive patients were included. 8 were female (40%), 12 (60%) were male. 12 (60%) underwent autologous transplants –10 for plasma cell dyscrasias, and 2 for lymphoma. 8, (40%) were allogeneic transplants (1 from a matched sibling donor and 7 from haploidentical related donors). Conditioning regimen included myeloablative (3,37.5%) and reduced intensity regimen (5,62.5%). The mean time to onset of oral mucositis (OM) was day +3.5 (range -1 to 7 days).

OM grade 1-2 was seen in 13 patients (65%), 6 (30%) had grade 3, 1 had grade 4 following total body irradiation. All patients used chlorhexidine mouth wash as prophylaxis. Patients receiving high dose melphalan received prophylactic cryotherapy with ice chips. 1 patient additionally had an oral cooling system deployed as a preventive measure.

6 (33%) patients required morphine, 6 (33%) required fentanyl boluses and 1 patient with grade 4 mucositis required a continuous IV infusion of fentanyl.

Severity of OM was not related to sex, age or BMI.

Mucositis resolved in a mean of 10.3 days (8-18 days). There was no significant difference in the time to onset / resolution of the mucositis between patients undergoing autologous or allogeneic transplants. RIC was associated with shorter mucositis duration though it did not reach statistical significance. TBI was associated with the only grade 4 mucositis.

Key findings:
· Severity of OM was not related to sex, age or BMI.

· Mean OM resolution time was 10.3 days (8-18 days).

· No significant difference in the time to onset of mucositis noted between patients undergoing autologous or allogeneic transplants.

· Autologous transplants and RIC were associated with shorter mucositis duration though not statistically significant.

· TBI was associated with grade 4 mucositis.

Conclusions:
Oral mucositis is a common and debilitating complication following stem cell transplant. This prospective study contributes to limited Indian data on the incidence, severity and risk factors for OM development. Contrary to previous studies, there was no association with female gender, age and lower body mass index. The small sample size limits statistical power.

Poster Presentation

Infectious and Non-infectious complications

Incidence of Pneumocystis jirovecii Pneumonia among Post-Allogeneic Haematopoietic Stem Cell Transplant Patients in Malaysia: A Single Center Study

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Background: Pneumocystis pneumonia (PJP) caused by the fungus *Pneumocystis jirovecii* may occur in allogenic haematopoietic stem cell transplant (HSCT) recipient and is associated with a high mortality rate. It occurs predominantly during the first nine months after transplant or later in the presence of graft-versus-host disease (GVHD). Even with effective prophylaxis, breakthrough infection may still occur. This study aimed to determine the incidence of PCP among allogeneic HSCT recipients in a single centre in Malaysia.

Methods: This was a retrospective study of patients who have undergone allogeneic HSCT from 1 January 2016 to 31 December 2024. Patient demographic and disease characteristics were obtained from medical records. Classification of PJP were defined based on consensus of European Organization for Research and Treatment of Cancer and the Mycoses Study Group Education and Research Consortium.

Results: A total of 77 patients were included in the study, consisting of 45 males and 32 females. The median age was 40 years, ranges from 18 to 64 years. The majority of patients were of Chinese ethnicity (n=46, 59.7%), followed by Malay (n=22, 28.6%). Acute myeloid leukemia was the most prevalent underlying condition (n=39, 50.6%), with acute lymphoblastic leukemia following as the second most common (n=16, 20.8%). Most of the patients (n=35, 45.5%) underwent haploidentical HSCT, followed by allogeneic HSCT (n=29, 37.7%), reduced intensity allogeneic HSCT (n=11, 14.3%) and matched unrelated donor allogeneic HSCT (n=2, 2.6%). The incidence of PJP was 18.2%, with 11 probable cases (78.6%) and 3 proven cases (21.4%). PJP developed after a median of 229 days, with 4 patients experiencing onset within 30 days post-HSCT. Among the 10 patients who developed PJP beyond the first 90 days post-HSCT, six had breakthrough infection despite co-trimoxazole prophylaxis. All of these patients were on immunosuppressant for GVHD. At diagnosis, only one patient had lymphopenia. The median absolute lymphocyte counts for patients who developed PJP beyond the first

90-days post-HSCT was $1.33x10^9$/L. Despite treatment, the 60-day mortality rate for PJP following allogeneic stem cell transplantation was 71.4%.

Conclusion: PJP in our cohort primarily occurred as a late complication following allogeneic HSCT. The high incidence of PJP in this study underscores the critical importance of PJP prophylaxis especially in patients receiving immunosuppressive therapy and illustrates the need of having high index of suspicion to prevent adverse outcome.

Poster Presentation

Infectious and Non-infectious complications

Use of pegylated G-CSF in autologous HSCT

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Infection complications are the main cause of mortality after ASCT. G-CSF are widely used after ASCT to shorten the period of aplasia.
In this retrospective study, we analyzed the results of applying the pegylated forms of G-CSF, Pegfilgrastim (6 mg) and Empegfilgrastim (7.5 mg), after ASCT.

Methods and materials: A total of 138 patients were enrolled in our research. 91 patients in the study group received a single infusion of Pegfilgrastim (n=81) or Empegfilgratim (n=10) the day after ASCT. In the control group, 47 patients underwent ASCT without G-CSF. The median age of patients and the amount of infused CD34+ cells were comparable between the study and control groups (55.9 (21-60) years vs 56.8 (39-69) years; 5.98*10^6 cells/kg(1.42-17) and 6.68*10^6 cells/kg(2.03-16.2)).
Multiple myeloma and lymphoma were the most common nosologies in both groups: study group: 64.8% and 35.2%; control group: 76.6% and 23.4%.
Patients with multiple myeloma received a conditioning regimen with Melphalan (40,4%) and Bendamustin+Melphalan (59,6%) in the study group and 30,5% and 69,5% in the control group. All patients with lymphoma proceeded ASCT with Benda-EAM conditioning.

Results: As expected, we observed a shortening of the period before neutrophil recovery in the study group (11.2 days (7-19) and 13.51 days (10-22), $p<0.01$). The time to platelet recovery, the median number of transfused doses of platelet concentrates, and Red Blood Cell units were similar between the study and control groups (12.6 vs 12.67; 3.8 vs 3.6; 0.53 vs 0.51, $p>0.05$).
The rate of all-grade oropharyngeal mucositis in the study and control groups was comparable (95.6% vs 97.8%, $p>0.05$); however, we found that the incidence of high-grade oropharyngeal mucositis (grades 3-4) was significantly lower in the G-CSF group (17.58% vs 38.29%, $p<0.05$). Additionally, the incidence of neutropenic enteropathy was significantly reduced in the G-CSF group for both all grades (74.72% vs 91.49%, $p<0.05$) and high grades (grades 3-4) (17.58% vs 38.29%, $p<0.05$).
We also observed a decrease in the frequency of febrile neutropenia in the G-CSF group (79.12 vs 93.6%, $p<0.05$), with no significant difference in the rates of sepsis

(10.9% vs 4.2%), bacteriemia(29.67% vs 25.5%), viral infections (17.58% vs 25%), fungal infections (46.1% vs 53.1%) or clostridial infections(3.3% vs 4.2%). Early transplantation related mortality was comparable between G-CSF and control groups (4.2% vs 2.2%).

Conclusion: Pegylated G-CSF (Pegfilgrastim and Empegfilgratim) is an appropriate alternative to the conventional form and may shorten the time to granulocyte recovery and reduce the incidence of mucositis and enteropathy after ASCT. This translates to a decrease in the rate of febrile neutropenia and could help reduce antibiotic use and the length of stay in the hospital.

Poster Presentation

Pediatric Transplantation

Good, Better And Best: A Twenty Year Follow Up Study Of Children With Severe Aplastic Anemia From A Single Centre In India

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Introduction:
Severe aplastic anemia (SAA) is a rare heterogenous group of disorder in children which can be complicated by life threatening infections, major bleeds and early mortality. Matched sibling donor hematopoietic stem cell transplantation (HSCT) is the treatment of choice. If a matched sibling donor isn't available, matched unrelated donor HSCT or immunosuppressive therapy with anti-thymocyte globulin have been the proposed lines of treatment. With the advent of haploidentical donor HSCTs, there has been a change in the way SAA is treated in the wake of life-threatening events. We present our experience of treating SAA over the past 20 years.

Patients and methods:
This is a retrospective study of children between 0-18 years of age with SAA who underwent treatment at our centre between January 2004 to December 2024. Prior to 2015, the treatment options were IST or matched donor HSCT and after that, haploidentical HSCTs were also offered to children with life-threatening SAA. The data was collected from the medical records and electronic data base. Appropriate statistical methods were used.

Results:
In the duration of 21 years, a total of 27 children underwent matched donor HSCT (unrelated donor = 3), 35 children underwent IST and 28 children underwent haploidentical donor HSCTs. The comparison of characteristics between matched donor and haploidentical HSCTs are show in Table 1**.** (*Table 1, attached at the time of submission, was deleted due to space limitations. —BCT Editorial Office*) The significant differences between the matched donor HSCTs and haploidentical HSCTs were donor age (median of 13 vs 38 years), graft rejection (0 vs 21.4%), CMV reactivation (0 vs 28%) ($p < 0.05$). The 5-years overall survival (OS) of the matched donor cohort

was 92.4% (95% CI: 73.06 - 98.06) vs haploidentical donor HSCT with 62% (95% CI: 38.7 – 78.6) vs 82.35% for IST (95% CI: 64.87 – 91.66) (p=0.04).

In the haploidentical cohort, among the patients who received rabbit ATG in the conditioning, 13/14 (92.8%) were alive which is on par with matched donor HSCTs with a follow up range of 7-54 months. The cause of death in that one child was transplant associated microangiopathy following a second HSCT. Among those who did not receive r-ATG in the conditioning, the graft rejection rate was 7/14(50%). Among the IST cohort, 5 underwent HSCT and 4 of them are alive and were included in the survival analysis of IST as well. Five relapsed patients in the IST cohort did not undergo HSCT and are alive on immunosuppressive medications. In the IST cohort, the survival was better among children who received IST with eltrombopag in comparison to IST alone i.e 94% (95% CI:66-99.2%) vs 68.75% (95% CI:40 – 78.3%) (p = 0.07).
The event free survival (EFS) among the three cohorts was 92.5% for matched donor HSCT vs 62.5% for haploidentical donor HSCT vs 44% for IST. The events recorded were relapse or death.

Conclusion:
Matched donor HSCT remains the treatment of choice for SAA. In the absence of a fully matched donor, haploidentical donor HSCT with r-ATG based conditioning is an equally good alternative over IST. Addition of eltrombopag improves efficacy of IST compared to IST alone.

Poster Presentation

Pediatric Transplantation

The Outcomes of Hematopoietic Stem Cell Transplantation in Children with Myelodysplastic Syndrome – A single centre study from India

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Introduction:
Myelodysplastic neoplasms in children are rare clonal hematopoietic disorders with heterogenous bone marrow cellularity, varied cytogenetics, germline predispositions and propensity to develop secondary to chemotherapy or inherited bone marrow failure syndromes (IBMFS). The 2022 WHO classification has simplified it into MDS without low blasts (< 5% bone marrow, <2% Peripheral blood) or excess blasts (5-19% BM, 2-19% PB). Hematopoietic stem cell transplant (HSCT) remains the only curative option, although various chemotherapy regimens are in vogue for its therapy. We present our data on HSCT in MDS.

Materials and Methods:
This is a retrospective analysis of children who underwent HSCT for MDS at our centre from January 2008 to December 2023. The data was collected from medical records and electronic data base. Data was analysed using appropriate statistical methods. Institutional ethics approval was obtained.

Results:
A total of 21 children underwent HSCT at our centre over 16 years for MDS with an age range of 8 months to 18 years. Male to female ratio was 2.5:1. Out of the 21 children, 3 (12.5%) children developed MDS secondary to chemotherapy, 3 (12.5%) children secondary to IBMFS (1 had Fanconi anemia and 2 children had MYSM1 gene mutation, 1 DBA). Monosomy 7 was noted in 4 (19%), whereas trisomy 8 and ANKRD26 germline mutation was noted in 1 child each. Five (24%) children transformed into acute myeloid leukemia (AML). Pre HSCT chemotherapy was administered in 8 (38%) children - Azacytidine in 3, ADE/HiDAC in 2, Lenalidomide in 2 children.

Matched sibling donors were used in 8 (38%), followed by haploidentical family donors in 7 (33%), matched unrelated donors in 6 (28%). Peripheral blood stem

cells were the commonest source of stem cells in 14 children (67%) followed by cord stem cells in 4 (19%). Fludarabine and busulphan was the commonest conditioning used in 6 (28.5%) children followed by Flu-Treo in 5 (24%) and Flu-treo-thio in 4 (19%). A total of eighteen (86%) children engrafted. Acute graft versus host disease (GVHD) was seen in 10/18 (55.5%) in which 3 children developed GVHD post pre-emptive donor lymphocyte infusions. Extensive chronic skin GVHD was seen in 2 (11%) and lung GVHD in 2 (11%) children. Viral reactivations were noted in 6 (28.5%). A total of 11 (52.3%) children were alive with a follow up range of 15 to 192 months**.** Cause of death was sepsis in 5/10 (50%) followed by disease relapse in 2 (20%).

Among the patients who transformed into AML, 2/5 (40%) were alive. In the cohort who received pre-HSCT chemotherapy, 3/8 (38%) were alive and 8/11 (73%) in those who did not. Among the patients with cytogenetic abnormalities, monosomy 7 had a poor outcome with 25% survival.

Conclusion:

The overall survival of children with MDS who underwent HSCT is 53%, with overall survival of 56% among those without transformation, versus 40% in those who had transformed to AML. Monosomy 7 had poor survival among the cytogenetic abnormalities. Myeloablative conditioning is essential to achieve engraftment with a sustained and durable graft.

Poster Presentation

Pediatric Transplantation

Outcomes of Pediatric Hematopoietic Stem Cell Transplantation in a Newly Established Tertiary Centre in a Tier-2 City in India: A Five-Year Retrospective Analysis

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Background:
Hematopoietic stem cell transplantation (HSCT) remains the cornerstone of curative therapy for various pediatric malignant and non-malignant hematological disorders. While transplant outcomes from major academic centers in metropolitan cities are well documented, limited data are available from emerging transplant programs in tier-2 cities. This study presents a five-year retrospective analysis of pediatric HSCT outcomes from a newly established transplant unit in a tertiary care center in South India.

Methods:
A retrospective review was conducted of all pediatric patients (≤18 years) who underwent HSCT between September 2019 and March 2025. Patient demographics, underlying diagnoses, type of transplant (autologous or allogeneic), conditioning regimens, stem cell source, engraftment kinetics, transplant-related mortality (TRM), and survival outcomes were analyzed. All patients received granulocyte-colony stimulating factor (G-CSF) mobilized peripheral blood stem cells. Engraftment was defined as the first of three consecutive days with absolute neutrophil count (ANC) ≥500/μL and platelet count ≥20,000/μL without transfusion support.

Results:
A total of 65 pediatric transplants were performed over five years, including 23 autologous and 42 allogeneic transplants. The median age was 10 years (range: 1–18), with a male-to-female ratio of 2.1:1.

Autologous Transplants (n = 23):

Indications included relapsed/refractory lymphoma (n = 12), neuroblastoma (n = 5), Ewing sarcoma (n = 3), CNS tumors (n = 2), and metastatic retinoblastoma (n

= 1). The median CD34+ cell dose infused was 7 × 10^6/kg. Median time to neutrophil and platelet engraftment was Day +10 (range: 9–16) and Day +15 (range: 14–18), respectively. At a median follow-up of 30 months, 19 patients were alive and disease-free, corresponding to an OS of 82.6%.

Allogeneic Transplants (n = 42):

Transplant types included matched sibling donor (MSD, n = 24), haploidentical (n = 16), and matched unrelated donor (MUD, n = 2) HSCTs. Indications were relapsed acute lymphoblastic leukemia (n = 21), relapsed acute myeloid leukemia (n = 5), and benign hematological disorders (n = 16), including aplastic anemia (n = 8), thalassemia major (n = 5), and pure red cell aplasia (n = 3). The median CD34+ cell dose was 6 × 10^6/kg. Neutrophil and platelet engraftment occurred at a median of Day +14 (range: 12–19) and Day +19 (range: 17–24), respectively. At a median follow-up of 28 months, survival was 75% for MSD transplants and 57% for haploidentical transplants.

Conclusion:

This single-center retrospective study highlights that pediatric HSCT can be performed with encouraging outcomes even in a tier-2 city setting. Autologous HSCT demonstrated excellent outcomes in relapsed solid tumors and lymphomas. Matched sibling allogeneic HSCT provided favorable survival in both malignant and benign hematologic conditions. Outcomes following haploidentical HSCT remain suboptimal, underscoring the need for further protocol refinement and enhanced supportive care. These findings support the feasibility, safety, and effectiveness of decentralizing pediatric transplant services to regional centers in resource-limited settings.

Poster Presentation

Pediatric Transplantation

Donor age and not donor specific antibodies scores above all other donor characteristics in children undergoing haploidentical hematopoietic stem cell transplantation with post-transplant cyclophosphamide in children for non-malignant disorders – a ten-year single centre follow-up study from India

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Background:
A haploidentical donor has a significant impact on the rate of infection, graft versus host disease, rejection and overall survival in children undergoing T replete haploidentical HSCT. There is limited data available on the impact of donor characteristics on the outcome of this group of children.

Patients and Methods:
We performed a retrospective analysis of patients who had been transplanted at our centre between 2015 to 2024 with a minimum follow up period of 6 months. We included all children had a T replete haploidentical HSCT with PTCY below age 18 years. We targeted a minimum CD34 cell dose of over 7 x 10^/kg and a CD3 dose of over 1.5 x 108/ kg in all our patients. Data collected included the patient diagnosis, age at HSCT, sex and conditioning regimen. The donor data collected included HLA disparity, donor age, sex mismatch, blood group incompatibility, the presence or absence of B leader mismatch and DSA positivity.

Results:
A total of 194 children underwent haploidentical HSCT with PTCY at our centre with male: female ratio of 1:1 with median age of 8.5 years (range 6 months – 18 years). The diagnosis was transfusion dependent thalassemia – TDT (90), inherited marrow failure syndrome – IBMFS (46), inborn error of immunity – IEI (31) and severe aplastic anaemia - SAA (27). The conditioning regimen was myeloablative in 55% (108/193) children and most patients had a peripheral blood stem cell source. The

donor was the father in 78% (151/194) children and in 62% (122/194) the donor was over 35 years of age. We documented blood group incompatibility in 41% (80/194) children and B leader mismatch in 26% (50/194) children. The graft failure rate was low at 10.2% (20/194). The incidence of acute graft versus host disease (a GVHD) was 53(27%) and limited chronic graft versus host disease at 19%(37/194).

HLA disparity, sex mismatch, donor-specific antibody (DSA) positivity, ABO incompatibility, and B-leader status did not significantly impact outcomes such as the incidence of infection, graft-versus-host disease (GVHD), rejection, or overall survival. However, a statistically significant correlation was observed between donor age and survival outcomes: recipients of grafts from donors younger than 35 years had significantly better overall survival compared to those with donors older than 35 years (p value 0.001)

The overall survival in the cohort was 75% as depicted in the Kaplan Meier curve. The most common cause of death was infection and children with IBMFS had a higher risk of GVHD.

Conclusion:

T cell–replete haploidentical hematopoietic stem cell transplantation with post-transplant cyclophosphamide provides a curative treatment option for children with benign hematologic disorders who lack matched family donors. Among all donor-related variables analysed, donor age emerged as the only factor with a statistically significant impact on survival outcomes. Children with donor specific antibodies can be taken up for HSCT as it did not influence transplant outcomes, neither when analysed by individual disease categories nor in the overall cohort.

Poster Presentation

Pediatric Transplantation

Excellent Thalassemia Free Survival In Haploidentical Hematopoietic Stem Cell Transplantation With Myeloablative Conditioning And Posttransplant Cyclophosphamide In Older Children With Antibodies And Transfusion Dependent Thalassemia A Retrospective Study Over An Eight Year Period

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Background:
Haploidentical hematopoietic stem cell transplantation (HSCT) for children with transfusion dependent thalassaemia (TDT) carries the risk of graft rejection, graft versus host disease and infections secondary to delayed immune reconstitution. We present our experience in haploidentical HSCT in children using posttransplant cyclophosphamide (PTCY) with a reduced conditioning (RIC) cyclophosphamide versus a myeloablative (MAC) treosulfan based conditioning.

Patients and methods:
We conducted a retrospective analysis at Apollo Cancer Centre, Chennai, Blood and Marrow Transplantation unit between January 2015 and October 2024.

Cohort 1 received a RIC conditioning with Fludarabine/Cyclophosphamide/Thiotepa and Cohort 2 received a MAC conditioning with Thiotepa/Treosulfan/Fludarabine. All children received two cycles of pre-transplant immunosuppression (PTIS) with Fludarabine/Dexamethasone and received rabbit anti-thymocyte globulin and 2 Gy total body irradiation as a single fraction during conditioning followed by post-transplant cyclophosphamide. We analysed the impact of the conditioning regimen on the engraftment, graft failure, sinusoidal obstruction syndrome, autoimmune haemolytic anaemia, viral reactivation, panel reactive antibody, the influence of PTIS, graft versus host disease, Thalassaemia Free Survival and Overall Survival.

Results:
We included 90 children in the study with 31 children in the RIC-Cohort 1 and 59 children in the MAC Cohort 2. The median age at transplantation was 8 years in Cohort 1 and 10 years in Cohort 2, with a similar male-to-female ratio of 0.7: 1. We documented a positive Panel Reactive Antibody (PRA) positivity in 40% in Cohort 1 and 66% in Cohort 2. Graft failure rates were higher with RIC at 25% versus 5% in the MAC group. Mortality was high at 9.6% in Cohort 1 and 15% in Cohort 2. Mixed chimerism higher in Cohort 1 at 28% versus 4%in Cohort 2. An adverse response to PTIS predicted survival. Children with platelet count of < 150,000 and absolute neutrophil count < 750 during PTIS had inferior survival and this was statistically significant with p value-0.01 and 0.02 respectively. PRA did not increase rejection rates. We documented an increase in survival in Cohort 2, with Thalassaemia Free Survival of 93% and Overall Survival of 86%, compared to 73% and 64% in Cohort 1 (p value at 0.002% and 0.006% respectively).

Conclusion:
In high-risk children with TDT older than 7 years with positive PRA and donor specific antibodies, it is possible to reduce rejection rates to 5% and offer Thalassaemia Free Survival of 93% using a Treosulfan based MAC conditioning. RIC protocol increases the risk of AIHA and late morbidity. We plan to reduce the Transplant Related Mortality (TRM) of 15% by reducing the dose of Thiotepa to 5mg/kg based on the cytopenia response with PTIS and aggressive therapy for adenoviral infection. This study offers hope to many children in low middle income countries with no matched donors and no access to in vitro T cell depleted HSCT.

Poster Presentation

Pediatric Transplantation

I-131 MIBG therapy Combined with autologous stem cell transplantation for high risk and Relapsed/ Refractory pediatric Neuroblastoma in MAHAK Hospital

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Introduction:
There are many different protocols for treatment of high risk and Relapse/ Refractory (R/R) Neuroblastoma. According to previous researches (safety, feasibility, phase I and phase II) I-131 MIBG therapy combined with high dose chemotherapy (HDC) and autologous hematopoietic stem cell transplantation (AHSCT) is an effective and promising (but not standard of care) method of treatment in these challenging patients.

Methods:
In 2 years, 18 pediatric patients (median age at diagnosis 46.5 months) after induction chemotherapy and surgical resection (at least good partially response patients) received I-131 MIBG therapy (18 mCi/kg) combined with (2-4 weeks later) HDC (busulfan/melphalan and CEM) and AHSCT. Two months later, six patients take local radiotherapy after AHSCT.

Results:
After 1 year follow-up 12 patients are alive (66.7 %) with complete response. We have two death due to relapse. 4 patient relapsed and alive after AHSCT. There are no major complications except severe prolong Thrombocytopenia in 5 patients and one death due to Veno-occlusive disease (VOD).

Conclusion:
This limited clinical trial show that I-131 MIBG therapy combined with HDC and AHSCT is safe and effective. In future, the results of ongoing randomized controlled trials determine the efficacy and exact role of this method of treatment.

Poster Presentation

Pediatric Transplantation

Outcomes of Eculizumab Use for Paediatric High Risk Transplant-Associated Thrombotic Microangiopathy in Hong Kong

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Aims:
Transplant-associated thrombotic microangiopathy (TA-TMA) is an under-recognized yet potentially devastating complication of hematopoietic stem cell transplantation (HSCT). Untreated high risk TA-TMA (hrTA-TMA) has dismal outcome due to multiorgan dysfunction. Eculizumab, a humanized monoclonal antibody inhibiting complement protein C5, has emerged as a first line targeted therapy for paediatric hrTA-TMA. We hereby summarise the treatment outcome of paediatric TA-TMA with eculizumab in Hong Kong.

Methods:
All patients aged below 18 years who underwent HSCT in the Hong Kong Children's Hospital and were diagnosed to have high risk TA-TMA according to Jodele 2014 criteria during the 6-year period from 1 April 2019 to 30 April 2025 were included.

Results:
A total of 226 HSCTs (173 allogeneic and 53 autologous) in 181 patients had been performed. 10 patients (four males and two females) developed high risk TA-TMA at a median duration of 1.6 months (range 0.25-5 months) post-HSCT. The incidence rate was 4.4%. Of the 10 TA-TMA patients, 8 underwent allogenic and 2 underwent autologous HSCT, respectively. Three of them were histologically proven. All five patients with calcineurin inhibitor had stopped the drug once TA-TMA was suspected. Genetic predisposition were identified in two patients. One had a variant of unknown significance in THBD gene [heterozygous NM_000361.3 (THBD):c.619G>A p. (Val231Ile)] presumably predispose patient to TA-TMA, while another patient had pathogenic variant in TP53 [heterozygous NM_000546.5(TP53):c.827C>A p.(Ala276Asp)] consistent with diagnosis of Li-Fraumeni syndrome likely unrelated to TA-TMA. All but the first patient (due to unavailability of the drug) were started on eculizumab immediately after diagnosis. Median 11 doses (range 5-13 doses) of eculizumab were administered. One out of

the nine patients had early termination of eculizumab due to severe sepsis (S. epidermitis bacteriemia, disseminated adenovirus and candida infection), while the rest stopped eculizumab when haematological TA-TMA and specific high risk feature resolved. Patients were put on prophylactic penicillin V from first dose of eculizumab till 3 months from last dose. 4 patients died (three due to fungal infection, one due to fungal and adenovirus infection) within 3 months upon diagnosis of TA-TMA. All of the death cases had concurrent GI bleeding, and persistent schistocytes, anaemia and thrombocytopenia despite eculizumab. Mortality rate was 40%. Among the 6 survivors, all had resolution of haematological parameters except residual chronic kidney disease (CKD). Three have mild stage 2 CKD and 3 with advanced stage CKD, including one stage 5 end-stage CKD requiring lifelong dialysis and one stage 4 CKD planning for renal replacement therapy.

Conclusion:
In conclusion, early recognition and prompt administration of complement blockage with eculizumab may be beneficial in selected cases. Fungal infection is a concern and major mortality. Further prospective research studies are recommended to improve the management and outcomes of TA-TMA.

Poster Presentation

Pediatric Transplantation

Haploidentical familial donor is a feasible option for childhood myelodysplastic syndrome

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Introduction: Allogeneic hematopoietic stem cell transplantation (HSCT) is the only curative treatment for childhood myelodysplastic syndrome (MDS), particularly effective for high-risk cases with monosomy 7, complex karyotypes, or advanced disease. It replaces defective marrow with healthy donor cells, offering 5-year overall survival rates of 64-94% for low-grade MDS and 30-63% for advanced MDS. Haploidentical HSCT shows comparable outcomes to matched donors, expanding donor options

Methods: We retrospectively analyzed the medical records of patients diagnosed with myelodysplastic syndrome (MDS) who underwent hematopoietic stem cell transplantation (HSCT) between 2015 and 2025 at the Department of Pediatrics, Pusan National University Yangsan Hospital.

Result: Of the 11 patients, 7 were male (63.6%) and 4 were female (36.4%). The mean age at diagnosis was 8.7 ± 4.6 years. The mean age at HSCT was 10.4 ±5.2 years. Donor types for HSCT included haploidentical family donors (HFD, 45.5%), matched unrelated donors (27.3%), matched related donors (18.2%), and mismatched unrelated donors (9.1%). The 5-year event-free survival (EFS) and overall survival (OS) rates were both 80.8%. OS was 53.3% for HFD and 100% for non-HFD donors ($p = 0.0771$).

Conclusion: Our findings suggest that HSCT using HFD is a feasible option for childhood MDS patients lacking an available matched donor.

Poster Presentation

Pediatric Transplantation

Outcomes of T-cell Receptor Alpha-beta (TCRab) Depleted Haploidentical Haematopoietic Stem Cell Transplantation with Donor Memory T-lymphocyte Infusion for Paediatric Acute Myeloid Leukaemia

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Aims:
Allogeneic hematopoietic stem cell transplantation (HSCT) is currently the standard therapy for paediatric high risk, refractory and relapsed acute myeloid leukaemia (AML). For patients lacking human leukocyte antigen (HLA)-matched family or unrelated donor, haploidentical HSCT is an increasingly used alternative.

Methods:
This is a retrospective study conducted in the Hong Kong Children's Hospital, the only paediatric HSCT center in Hong Kong, during 1 March 2021 to 31 December 2024. Paediatric patients aged 18 years or below with high risk, refractory or relapsed AML indicated for allogeneic HSCT and underwent haploidentical HSCT with T-cell receptor alpha-beta (TCRab) depleted peripheral blood stem cell (PBSC) graft were included. Conditioning regimen consisted of total body irradiation (TBI) 3Gy in single fraction on day -8 (before June 2023) or total lymphoid irradiation (TLI) 6Gy in 3 fractions (2Gy twice daily on day -9 then 2Gy daily on day -8)(in or after June 2023), intravenous fludarabine (Flu) 30mg/m2/day for 5 days on day -7 to -3, intravenous thiotepa (TT) 5mg/kg/dose every 12 hours for 2 doses on day -3, and intravenous melphalan (Mel) 70mg/m2/day for 2 days on day -2 and -1. Filgrastim-mobilized PBSCs were collected through apheresis and transplanted fresh after TCRab-depletion with targeted cell dose of 10 x106 CD34+cells/kg (minimal 5 x106 CD34+cells/kg). Donor memory T-lymphocytes were also infused fresh on HSCT day 0 after CD45RA (before September 2023) or CD62L depletion (in or after September 2023), with targeted cell dose of 1 x 106 CD3+cells/kg. No routine pharmacological prophylaxis for graft-versus-host disease (GVHD) was given.

Results:
Total 10 patients were included (4 males and 6 females), with median age of 11.7

years (range 7.3-18.2 years). Eight patients had de novo AML, 1 patient had therapy-related AML and 1 patient had AML with underlying myelodysplastic syndrome. Nine patients had morphological complete remission (CR) prior to HSCT (5 in CR1 and 4 in CR2), while the remaining 1 patient was not in remission after relapse at the time of HSCT. Measurable residual disease (MRD) was positive in 4 of the 9 CR patients (range 0.48-3.9%). 8 out of 10 patients employed haploidentical donor that was favourable in killer immunoglobulin-like receptors (KIR)(4 with donor KIR : recipient ligand mismatch; the other 4 with donors B-content score of 2 or above). The remaining 2 donors were KIR-unfavourable due to lack of availability of alternative donors. Median neutrophil and platelet (≥20x109/L) recovery were on day +10 (range 9-11) and day +9 (range 8-17) respectively. All patients achieved morphological and molecular remission with full donor chimerism at 1 month post-HSCT. One-year overall survival and leukaemia-free survival were 100% with median follow-up of 16.5 months (range 6-51 months). Two patients had acute GVHD (grade 2 and grade 3) and 1 patient had chronic lung GVHD.

Conclusions:
TBI/TLI-Flu-TT-Mel followed by TCRab depleted haploidentical PBSCT with donor memory T cell infusion is a promising approach even in MRD positive AML patients. Choosing a KIR- favorable donor may contribute to satisfactory outcome.

Poster Presentation

Pediatric Transplantation

TCR αβ/CD19 Depleted Haploidentical Hematopoietic Stem Cell Transplantation with CD45RO Add Back in Children: A Prospective Single-Centre Study from India

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Introduction:
Hematopoietic stem cell transplantation (HSCT) remains a curative option for many children with malignant and non-malignant disorders. Haploidentical donor HSCTs are lifesaving procedures for many children without matched sibling or unrelated donors. However, this comes with an increased risk of graft versus host disease(GVHD) and delayed immune reconstitution. TCR αβ/CD19 depletion is one such technique of removing the GVHD causing TCR αβ T-cells. One way of mitigating the delayed immune reconstitution is to add back memory T cells (CD45RO+). We present early results from a prospective single-centre study in children from India.

Patients and methods:
This is a prospective analysis of children (0-18 years) undergoing TCR αβ/CD19 depleted haploidentical HSCT for various malignant and non-malignant disorders at our centre. TCR alpha beta depletion and CD45RO+ collection is done using CliniMACS prodigy platform. CD4 and B cell counts will be measured at day +30, 60, 90, 6 months and 1 year from the date of transplant for assessing immune reconstitution. Cytomegalovirus(CMV), adenovirus and Epstein-barr viral loads by multiplex quantitative polymerase chain reactions(PCR) will be measured weekly from engraftment till day+100.

Results:
Eight children had been enrolled so far into the study, with an age range of 2 months to 8 years with 5 boys and 3 girls. There were 2 children each with mucopolysaccharidosis, 2 with juvenile myelomonocytic leukemia, 2 IEI, one child with inherited bone marrow failure and one with severe aplastic anemia. The median donor age was 33 years (4 years to 44 years) with 7 male and 1 female donors. All but one recipient & donor pairs were CMV IgG positive.

The average CD34 dose was $10x10^6$ cells/kg. All the patients got CD45 RO+ add back of $1x10^6$ cells/kg on D+7. Median day of neutrophil engraftment was D+13. Median CD4 count at D+30 was 39 cells/mm^3 (range: 0-103 cells) and 130 cells/mm^3 at D+60 (range: 4-183 cells/mm^3). The median absolute lymphocyte count at D+30 was 626 cells/mm^3 (51 – 1680 cells/mm^3) when compared to historical controls where 55% had an ALC below 500 cells/mm^3. One child with HLH had a mixed chimerism and graft rejection. Follow up duration is 28 days to 162 days.

Viral reactivation was seen in 5 children (62.5%) at a mean of D+20. CMV was reactivated in 4 children and adenovirus in 2 children which were managed with ganciclovir and cidofovir. A total of 7 CD45 RO+ add backs were given in 6 children at a median of D+32. Four (50%) of them received a stem cell top up infusion. All of them were alive at the time of the study with no grade (III-IV) GVHD and active viral reactivations.

Conclusion:
CD 45RO+ T-cell add back post TCRαβ depleted haploidentical HSCT in children is a feasible option. The add back helped with immune reconstitution and didn't increase the risk of GVHD. We intend to do a national level multicentric study in children with inborn errors of immunity on the similar protocol.

Poster Presentation

Cell and Gene Therapy

A COMPASSIONATE SINGLE CENTER, SINGLE ARMED, OPEN LABELLED TRIAL TO ASSESS THE EFFICACY AND SAFETY OF CHIMERIC ANTIGEN RECEPTOR (CAR)-T CELL THERAPY FOR PATIENTS WITH RELAPSED OR REFRACTORY AGGRESSIVE B-CELL LYMPHOMA IN MALAYSIA

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Introduction: Relapsed or refractory Diffuse large B cell Lymphoma (DLBCL) remains a big challenge to clinical hematologist. Up to 30% from newly diagnosed DLBCL will be complicated with failure of first line therapy. Salvage therapy options provide response rate around 40 to 60% and inversely proportional with subsequent lines of salvage chemotherapy. Therefore, there is still an unmet need for these patients and CAR-T is a new options viable, especially for those patients who were heavily pretreated with multiple lines of therapy. Locally manufactured CAR-T can help CAR-T affordable and accessible. Thus, this Phase Ib/II trial mainly aims to evaluate the efficacy and safety of locally produced-CAR T-cell therap. This study also aims to provide clinical basis and experience for CAR T-cell technology in the treatment of DLBCL in Malaysia, and most importantly, to provide an effective and accessible treatment to relapsed/refractory DLBCL patients.

Methods: This investigator-initiative, compassionate use trial is a single-centre, open-label, single-armed study to assess the safety and efficacy of CAR-T immunotherapy targeting CD19 and CD22 in subjects with relapsed or refractory aggressive B-cell lymphoma who have failed at least 2 lines of therapies. Conditioning chemotherapy comprised of fixed dose of 30mg/m2/day IV Fludarabine and 500mg/m2/day IV Cyclophosphamide followed by a single infusion of AUXICART at a target dose of 2.0 to 3.0 x 10^6 cell anti-CD19/22 per kg body weight. Primary end point was the percentage of patients with an objective response (complete or partial response), assessed by an independent radiologic review committee according to the Lugano classification. As per protocol, the primary efficacy analysis was conducted at cutoff date of 28/02/25.

Result: A total of 25 patients were enrolled. AUXICART 19/22 was manufactured for 22 patients and administered to 13 patients. 2 patients were not assessable due to disease progression on the second day post infusion and needed urgent salvage chemotherapy. Baseline characteristic showed median 3 prior lines of therapy (range 2 to 5) and 36.4% have received autologous stem cell transplant. The primary efficacy analysis showed that 36.4% of the 11 patients had an objective response, 9.1% achieved complete response. Median follow up is 10.5 months (range 2.1 to 17.2). Median survival duration is 8.5 month. At 10 months, estimated progression free survival is 38.5%. Longest survival achieved up to 14 months. 2 patients (18.2%) succumbed due to infection. 9.1% developed CRS (grade II) and 18.2% developed ICANS. Thrombocytopenia occurred at median time of 8 days post infusion with average of 96 x 10^9 /L(6 – 141x 10^9 /L), while neutropenia happened at 7 days post infusion, with average of 0.49 x 10^9 /L(0.03 – 1.89 x 10^9 /L).

Conclusion: Locally produced CART would be a promising option for heavily pretreated aggressive B cell lymphoma in our country, in improving their survival with acceptable toxicity profile. Perhaps the outcome could be better if CART introduced earlier in their disease course.

Poster Presentation

Basic Science

Data Management in Bone Marrow Transplant and Cellular Therapy

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Introduction: Data from medical records play a pivotal role in improving outcomes, identifying areas for improvement in program operations, and understanding the trends of pre- and post-interventional management. In Bone Marrow Transplant and Cellular Therapy (BMT&CT), cross sectional and longitudinal data help to understand the most efficient way of patient work-up, stem cell harvesting, stem cell infusion, post-transplant management, and patient outcome improvement. Data is being used in regulatory compliance for program accreditation, center of excellence qualification for insurance or payers, information for general people (public domain data), and, most importantly, for research & development, quality improvement, and publication.

Objective: The main objective of this presentation is to share the understanding for the management and utilization of clinical and administrative data in BMT&CT

Methodology: This study evaluated the Center for International Bone Marrow Transplant and Research (CIBMTR) data-reporting process with regulatory and IRB compliance , as well as, reviewed different forms that were used in the electronic data capturing (EDC) system of CIBMTR, recipient and donor data, Interphase for EDC, workflow, and capturing data using regulatory compliance. This study reviewed the data submission process for the insurance company and the general public. This presentation will also highlight the process of using data in departmental use for clinical trials, observational studies, and quality improvement in the administrative and clinical services.

Discussion: In the USA, most centers continuously collect and submit real time data to the CIBMTR database for regulatory compliance and for accreditation. Data for the insurance is collected by American Society for Transplant and Cellular Therapy (ASTCT) through surveys. Transplant data for the public knowledge is primarily coordinated and published through the organization like BMTInfo Net. Data for research, quality improvement, publication and presentation at the professional

conferences are organized by the departments. There are several other organizations who are involved in managing and using BMT&CT data.

Centralized multicenter data collection, like CIBMTR, help in standardizing the data elements for many transplant centers thus warranting validity, integrity and consistency of data. Which ultimately enables comparability across the centers. The important elements for transplant data are patient demographics, disease profile, courses of treatment, transplant procedures, and follow-up. The CIBMTR limits data errors and validity within a set standard level through mandating internal data audits and assessments by a separate individual.

The major challenges encountered in data management by the transplant centers are incomplete data due to patient lost in follow up, limited resource for data collection and management, and data transfer from source documents like electronic medical/health record (EMR/EHR) to data-reporting databases or EDC.

Conclusion: Well organized data collection and efficient data management can ensure enhanced patient care, administrative management, quality assurance and improvement, accurate information dissemination for patients, regulatory compliance, research and development and nurturing novelty in the field.

Poster Presentation

Basic Science

Use of Pegylated GCSF for stem cell mobilization in healthy donor for allogeneic stem cell transplant

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Aims & Objectives: To evaluate the safety and efficacy of pegylated granulocyte colony stimulating factor in mobilizing stem cells of donors for allogeneic stem cell transplant

Patients / Materials & Methods: We retrospectively evaluated data of 42 patients and their donors, whose stem cells were collected between January 2024 and February 2025. All the donors were healthy and received subcutaneous 6 mg of pegylated granulocyte colony stimulating factor and harvesting was done on day 5. In cases where the yield was less than the desired value, collection was attempted on Day 6 also.

Results: 95% (n=40) of the donors achieved the desired stem cell yield on Day 5, while the remaining 5% (n=2) required the apheresis process on the next day also. The failure rate was 0% on day 6. 39 (93%) out of 42 donors had skeletal pain after Peg GCSF and were managed with NSAIDs. No severe adverse event was seen. 3 donors required plerixafor for stem cell mobilization.

Discussion and Conclusion: Stem cell collection for allogeneic stem cell transplant after the administration of pegylated granulocyte colony stimulating factor is feasible and safe. Skeletal pain was the most common symptom and was well manageable.

Poster Presentation

Basic Science

Matching Probabilities of Eight Indian Population Groups in the Donor Pool of DKMS Foundation India

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Aims:
Allogeneic hematopoietic stem cell transplantation (HSCT) is often the last curative option for patients with severe hematopoietic diseases. Optimal transplantation results are achieved when patient and donor are matched regarding their human leucocyte antigen (HLA) genes. As in many cases no suitable related donor can be found, registries for voluntary unrelated donors have been established worldwide. HLA allele and haplotype frequency distributions are population-specific and therefore the probability of finding a matched donor for a patient depends on the ethnic composition and size of the donor pool. DKMS Foundation India is a Bangalore-based donor registry with nationwide donor recruitment activities. To evaluate the benefits of the registry's current donor pool for Indian patients and the need for future donor recruitment, we analyzed a large dataset of *n*=130,518 registered potential stem cell donors.

Methods:
We defined 8 subpopulations by combining information on the geographical origin and native language of both parents of the donors (native language: Kannada/state of origin: Karnataka, Tamil/Tamil Nadu, Telugu/Andhra Pradesh, Malayalam/Kerala, Bengali/West Bengal, Gujarati/Gujarat, Hindi/Uttar Pradesh and Marathi/Maharashtra) with sample sizes between *n*=5,808 and *n*=14,866. Based on the respective population-specific 5-locus (HLA-A, -B, -C, -DRB1 and -DQB1) haplotype frequencies, we estimated the probabilities for patients from these different populations to find a matched donor in their own donor pool or in the donor pool of the growing DKMS Foundation India.

Results:
The estimated matching probabilities reflect the intra- and inter-population HLA diversity of the different Indian populations. Highest 10/10 matching probabilities (MP; matched in 10 out of 10 HLA alleles) between 33.3% and 42.2% (both at a registry size of n=100,000) were reached by a donor search in the own population. A search in a donor pool of the current ethnic composition of the DKMS Foundation

India registry yielded 10/10 MP between 19.1% and 22.6% at the same registry size. The permission of a single mismatch (≥9/10 MP) between patient and donor in the scenario with identical patient and donor population increased MP to values between 60.2% and 69.4%. Patients from Southern Indian populations are the main beneficiaries of DKMS Foundation India registry in its current composition.

Discussion:

Our results support DKMS Foundation India's efforts to achieve nationwide coverage by opening local donor recruitment offices in different parts of India. Acceptance of singular mismatches in donor selection facilitated by the use of cyclophosphamide (PTCy)-based prophylaxis for graft-versus-host disease (GVHD) in unrelated mismatched HSCT may provide an option for patients from populations with high genetic variability and, more generally, from populations that are underrepresented in global volunteer donor registries.

Poster Presentation

Basic Science

Efficacy Of Allogeneic Stem Cell Transplantation In Acquired Aplastic Anemia: A Retrospective Single-Center Study at Blood Transfusion Hematology Hospital

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Background: Acquired aplastic anemia (AA) is a life-threatening hematologic disorder characterized by bone marrow failure. Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is an effective option for curative treatment.

Aims: This study aimed to evaluate the clinical characteristics, treatment outcomes, and prognostic factors associated with survival in AA patients undergoing allo-HSCT at the Blood Transfusion Hematology (BTH) Hospital from March 2008 to January 2025.

Methods: A retrospective cohort study was conducted on 54 patients diagnosed with AA who underwent HSCT. Data on demographic characteristics, treatment regimens, complications, and survival outcomes were collected and analyzed. Key outcomes were overall survival (OS), failure-free survival (FFS), graft failure-free survival (GFFS), and factors influencing these outcomes. Statistical analyses were performed using Kaplan-Meier survival curves and Cox proportional hazards models, with significance set at $p < 0.05$

Results: The median age at diagnosis was 21.35 years (range: 8.75 – 33.73), with a female-to-male ratio of 1.3:1. Peripheral blood stem cells were collected in all cases, with 98.2% of donors being related. The median follow-up was 47 months. The estimated 4-year OS, FFS, and GFFS rates were 92.2%, 88.3%, and 77.5%. The median CD34+ cell dose was 7×10^6/kg. Median neutrophil and platelet engraftment occurred at 15 and 19.5 days, respectively.

Patients receiving more than 50 units of pre-transplant blood transfusions had significantly lower OS ($p = 0.003$). Pre-transpant infections were also associated with reduced survival outcomes ($p = 0.003$). Post-transplant complications were

common, with 98.1% experiencing infections, including sepsis (31.5%) and pneumonia (16.7%). Cytomegalovirus (CMV) reactivation occurred in 61.1% of patients. Acute GVHD developed in 16.7% of cases, while chronic GVHD occurred in 25.9%, primarily mild (92.8%). The use of a Fludarabine-based conditioning regimen and rabbit ATG was associated with reduced rates of acute GVHD ($p = 0.009$, HR = 9.55; $p = 0.03$, HR = 4.2, respectively). Graft rejection occurred in 7.4% of patients. Therapy related mortality was 7.4%, with infections caused by multidrug-resistant organisms and pre-transplant infection significantly increased mortality risk ($p < 0.05$).

Conclusions: Allo-HSCT is an effective treatment for acquired AA, demonstrating favorable survival outcomes and manageable complications in the Vietnamese population. Conditioning regimens incorporating Fludarabine and rabbit ATG are associated with lower GVHD rates and may improve transplant outcomes. Optimizing infection prevention and limiting transfusion dependence pre-transplant are critical for enhancing survival.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Nursing care of a case of acute myeloid leukemia undergoing secondary allogeneic hematopoietic stem cell transplantation complicated with perineal herpes simplex virus type 2

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Objective: To summarize the nursing experience of a patient with acute myeloid leukemia (AML) who underwent secondary allogeneic hematopoietic stem cell transplantation (allo-HSCT) and developed herpes simplex virus type 2 (HSV-2) in the perineum.

Method: The patient, a 25-year-old female, was diagnosed with AML, which relapsed after allo-HSCT. On April 2, 2021, she was admitted to our hospital for a second allo-HSCT. On April 20th, multiple warts were observed in the perineum and perianal area. On April 28-29, the right labia majora ruptured and was treated with moist burn ointment.

On April 30, the perianal mucosa was damaged, and Beifuji spray was added to the perineum and perianal area. On May 20th, the patient was successfully discharged from the warehouse. A 2cm x 2cm scab was observed on the pubic mound, and a white attachment of 1cm x 3cm was found on the right labia minora. The right labia majora was red and swollen, with slight tenderness and a 1cm x 3cm scab. The anal fissure was located at the 12 o'clock direction of the anal lithotomy. A specimen was collected from the perineum, and the results were reported as positive for HSV-2, with Escherichia coli (+) and multiple drug resistance. Nursing measures: Use iodine solution for perineal and perianal care, rinse the affected area with physiological saline until dry, wet apply rehabilitation solution for 10-20 minutes, dry with oxygen, spray with Beifuji oxygen, alternate application of Shiwei Huangjin ointment and Meibao, apply acyclovir cream to warts, and apply Jinfu Ning to perineum and other ruptured areas to promote wound healing. Wet apply Kangfuxin liquid, apply magnesium sulfate for 20-30min to redness and swelling, and infrared radiation for 30min. On June 9, apply human interferon a2b gel to the wart and massage it locally, four times a day, and the damaged area around the anus begins to heal.

Results: After the application of human interferon a2b gel, the swelling, ulceration and perianal area of the perineum improved significantly, and a small amount of crusts began to fall off. On June 12th, most of the scab fell off and the perianal area was basically healed. On June 13th, the scab completely fell off and the ulcer was basically healed. On June 15th, the redness and swelling of the labia majora improved, the ulceration healed, the perianal area improved, and there was no pain after defecation. On June 18th, the patient tested negative for Escherichia coli.

Conclusion: In the care of patients with skin and mucosal damage after transplantation, correct evaluation, joint diagnosis and treatment by a multidisciplinary team, retention of imaging data for comparison and summary, digitization of damaged/foreign body area. During the nursing process, be good at observation, early detection, timely handling, dynamically observe the effect of medication, further improve nursing measures, and promote recovery. After transplantation, various types of skin are diverse and complex. The joint efforts of doctors, nurses, patients, family members, and their families can alleviate pain and promote patient recovery.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Case report: pseudotumor cerebri and hypercalcemia associated with the interaction between all-trans retinoic acid (ATRA) and voriconazole in an acute promyelocytic leukemia (APL)

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Aim:
This study aims to describe a unique case of pseudotumor cerebri and hypercalcemia resulting from an interaction between all-trans retinoic acid (ATRA) and voriconazole in a patient with acute promyelocytic leukemia (APL).

Case:
An 18-year-old female newly diagnosed with acute promyelocytic leukemia was admitted to the chemotherapy ward and began treatment with all-trans retinoic acid (ATRA) at a dose of 45 mg/m^2 with 1000 mg/day of hydroxyurea (day 1 to 17) and arsenic trioxide (ATO) 0.15 mg/kg (day 18-21). On the seventh day of ATRA therapy, she developed fever and later diagnosed with intramuscular abscess at her right calf and possible invasive pulmonary aspergillosis (IPA). Voriconazole therapy was initiated afterward concurrently with the same dose of ATRA. By the third day of voriconazole treatment, the patient complained of severe headache, bilateral orbital pain, tinnitus, and horizontal binocular diplopia. Neurologist and ophthalmologist were consulted which led to a diagnosis of pseudotumor cerebri. The MRI brain and lumbar puncture results were normal without any evidence of disease or infection involving the central nervous system. All symptoms of pseudotumor cerebri resolved 2 weeks after the discontinuation of both ATRA and voriconazole.

In addition, the patient had an increased in serum calcium level developed 20 days after ATRA treatment, with maximum level of 12.96 mg/dL. Her total 25-OH vitamin D level was low (15.10 ng/mL) and parathyroid hormone (PTH) levels was also very low at the level of 3.7 pg/ml (normal range 15 – 65 pg/mL). The alkaline phosphatase level was also elevated which reflected the increase in bone turnover rate. Four days after discontinuing ATRA and 2 days after stopping voriconazole, her

calcium level returned to normal with concurrent treatment using calcitonin, vitamin D_2, and zolendronic acid.
Bone marrow morphological at 24-day post ATRA induction showed no evidence of abnormal promyelocytes as well as a negative result for quantitative PCR for PML-RARA fusion gene. ATRA was restarted at 50% dose reduction and combined with ATO as the consolidation phase regimen for APL. Full dose of ATRA was achieved at day 11 after the re-challenge and the patient was discharged without any further complication.

Conclusion:
Oriconazole which is one of the strong CYP3A4 inhibitor, can elevate the serum concentration of all-trans retinoic acid (ATRA) when use concomitantly in patient with acute promyelocytic leukemia. This treatment in the setting of invasive fungal infection such as IPA can potentially lead to increase in ATRA-associated toxicities. This case report highlights the occurrence of pseudotumor cerebri and hypercalcemia in APL patient undergoing concurrent treatment with ATRA and voriconazole.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Gut Microbiome Disruption and Immune Reconstitution Following Haploidentical Stem Cell Transplantation with Post-Transplant Cyclophosphamide

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Background: Haploidentical transplantation with post-transplant cyclophosphamide (haplo-PTCy) is widely used for allogeneic hematopoietic stem cell transplantation (allo-HSCT). Given the unique nature of haplo-HSCT with PTCy, it remains unclear how high-dose cyclophosphamide might influence the composition of the microbiota and subsequent transplant outcomes. This study aims to investigate the impact of high-dose cyclophosphamide on microbiota composition and transplant outcomes in haplo-HSCT compared with matched related or unrelated transplants.

Methods: This study aims to comprehensively investigate the impact of high-dose cyclophosphamide on microbiota composition and subsequent transplant outcomes in haplo-HSCT compared with matched related or unrelated transplants. We analyzed the stool samples and immune profiles of 29 transplant patients (20 haplo-PTCy, nine matched donor) undergoing allo-HSCT at the China Medical University Hospital. Samples were collected pre-conditioning and 21–35 days post-transplant. Microbial composition was assessed using 16S rRNA sequencing, and immune reconstitution was evaluated by flow cytometry.

Results: Haplo-PTCy patients exhibited more pronounced reductions in gut microbial diversity post-transplant compared with matched donors. We also found unique bacterial populations, notably *Enterococcaceae* and *Erysipelotrichaceae*, the haplo-PTCy group. Functional analysis revealed increased glutathione S-transferase activity in the haplo-PTCy microbiomes. Immune reconstitution analysis showed distinct T-cell subset distributions between groups, with haplo-PTCy patients displaying higher proportions of CD8+ effector memory T cells, CD4+ regulatory T cells, and CD8+ PD1 + T cells. A positive correlation was observed between regulatory T-cell reconstitution and gut *Enterococcaceae* abundance.

Conclusions: This study reveals significant implications for allogeneic hematopoietic stem cell transplantation, particularly in haploidentical transplantation with post-transplant cyclophosphamide (haplo-PTCy). The observed differences in gut microbial composition and immune reconstitution between haplo-PTCy and matched donor transplant recipients highlight a complex interplay between transplant modality, microbiome dynamics, and immune system recovery. The haplo-PTCy group showed a more pronounced reduction in gut microbial diversity and unique bacterial populations, suggesting stronger selective pressures on the intestinal microbiome. Increased glutathione S-transferase activity in haplo-PTCy microbiomes indicates an adaptive response to high-dose cyclophosphamide, potentially affecting drug metabolism and toxicity management. Distinct immune reconstitution patterns in haplo-PTCy patients could have important implications for post-transplant outcomes and patient care strategies..These findings highlight the complex interplay between transplant modality, microbiome dynamics, and immune system recovery in allogeneic hematopoietic stem cell transplantation. The study underscores the potential impact of the haplo-PTCy approach on both the gut microbiome and immune reconstitution patterns.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Parkinsonism following CAR-T therapy in a patient with refractory multiple myeloma: a case report

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Case presentation:
A male in his 50s was diagnosed with multiple myeloma in September of year X. He underwent a total of seven courses of chemotherapy, but while some lesions disappeared, the overall trend was progressive. In April of year X+1, he was readmitted for CAR-T therapy, and Ide-cel was infused (day 0). On day 10, he developed cytokine release syndrome (grade 2), but his symptoms improved with tocilizumab administration. He was discharged home on day 25. However, due to the subsequent development of parkinsonism, which was thought to be related to CAR-T therapy, he was readmitted on day 39. A DaTscan performed after admission showed a mild decrease in the left striatum, predominantly in the putamen. Physical and occupational therapy were prescribed on day 47.

Assessment Results and Problems:
At the initial assessment (days 47–49), masked facies, tremor, and rigidity predominantly in the right upper limb were observed, with muscle tone graded as MAS 1 to 1+. Physical function was as follows: grip strength, 24 kg; 10-meter walking speed, 12.6 seconds; Short Physical Performance Battery (SPPB), 8 points; Timed Up and Go (TUG), 12.3 seconds; and Berg Balance Scale (BBS), 50 points, indicating an overall decline in physical function. Upper limb motor function, assessed using the Simple Test for Evaluating Hand Function (STEF), was 78 points on the right and 87 points on the left, demonstrating right-side dominant motor impairment. Activities of daily living (ADL), measured by the Barthel Index (BI), was 75 points.

Intervention and Results:
Due to significant fatigability, physical therapy focused on low-intensity aerobic exercise and muscle strengthening to maintain physical function. During hospitalization, daily oral steroids and four intrathecal steroid injections were administered by day 67. By discharge (day 93), grip strength improved to 28 kg, 10-meter walking speed to 9.5 seconds, SPPB to 12 points, TUG to 11.1 seconds, and BBS to 56 points, while STEF showed no significant change (right, 76 points; left, 88 points). ADL improved to BI 90 points.

Discussion:

Parkinsonism following CAR-T therapy is rare, and the characteristics of associated symptoms and physical function remain unclear. This patient exhibited right upper limb–dominant parkinsonism, which persisted despite treatment. Although physical function improved, the necessity for ongoing follow-up was indicated.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Development of Clinical Assessment Guideline for Cancer-Related Fatigue in Hospital

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Background/Aim:
Hematology oncology and bone marrow transplant patients often suffered from fatigue and decreased physical strength during disease progression, prolonged hospitalization, chemotherapy, bone marrow transplantation, radiation therapy, or immunotherapy. This condition is defined as cancer-related fatigue(CRF). In 2022, used brief fatigue inventory-Taiwan form(BFI-T) to assess 30 patients in the hematology-oncology and bone marrow transplant ward, showed 100% of the patients suffered from cancer-related fatigue. However, it was found that the hospital lacked standardized clinical guidelines for assessing cancer-related fatigue, prompting the initiative to develop guideline in September 2023.

Methods:
The author conducted a search across various databases and clinical guidelines from major oncology societies. A guideline development team was formed, consisting of oncology clinical nursing experts and nursing education specialists. The appraisal of guidelines for research and evaluation tool(AGREE II) was used for quality assessment by expert review. Guidelines from the Taiwan Oncology Nursing Society(TONS), National Comprehensive Cancer Network(NCCN), and European Society For Medical Oncology(ESMO) were referenced in drafting and integrating guideline recommendations. This guideline adopts the evidence grading and recommendation strength of the Joanna Briggs Institute (JBI). Upon completion of integration and drafting, an expert consensus meeting was be conducted where experts will collectively present their opinions on the guideline. Finally, revisions were completed following the consensus guidelines.

Results:
After the guidelines were completed, they were provided to the hospital's oncology team for clinical application. Nurses will conduct regular screenings according to the protocol and provide health education. Patients suffer from cancer-related fatigue will receive nursing interventions.

Conclusions:

The development of clinical assessment guideline requires following a standardized development process, which is quite complex and intricate. High-quality research evidence is essential for developing clinical guidelines that healthcare professionals can use to enhance patient care. This guideline aims to promptly identify whether patients are suffering from cancer-related fatigue and provide early treatment to alleviate the discomfort it causes.

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

Exploring Fertility Preservation Decision-Making and Coordinator Support Challenges in Female Patients Undergoing Hematopoietic Cell Transplantation

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Aims:
Fertility preservation (FP) is recommended for adolescent and young adult (AYA) patients undergoing hematopoietic cell transplantation (HCT). However, Hospital A lacks an in-house FP system, forcing patients to visit external reproductive clinics under time pressure. This study aimed to clarify how female patients made FP decisions in such circumstances and examine support challenges faced by transplant coordinators (HCTCs).

Methods:
We retrospectively analyzed two female patients in their twenties referred from Hospital A to reproductive clinics outside the prefecture. Based on HCTC counseling records, the FP decision-making process was examined across three phases:
I) Diagnosis to reproductive consultation
II) Consultation to FP decision
III) Post-HCT long-term follow-up
HCTC support in each phase was qualitatively analyzed.

Results:
Patient A (unmarried) chose to preserve her oocytes, while Patient B (married) declined FP.From diagnosis to reproductive consultation, both patients experienced anxiety about prognosis, burdens of travel under immunosuppression, and financial difficulties. HCTCs provided psychological support and coordinated clinic visits. From consultation to decision, both patients understood the medical and psychosocial issues associated with FP and post-HCT pregnancy. However, time constraints made decision-making difficult. HCTCs facilitated family discussions and reflection. Patient A chose FP with future marriage in mind, while Patient B decided against FP after discussions with her spouse.During long-term follow-up, FP-related support continued. Patient A, now married, saw her frozen oocytes as hope for future childbearing. Patient B and her partner faced infertility and required psychological support.

Conclusions:

For female HCT patients, fertility preservation is not merely a medical choice, but a life-defining decision reflecting personal values and life perspectives. Even post-decision, patients may face emotional conflict due to psychosocial changes and external influences. HCTCs must stay involved throughout the transplant process, providing support aligned with evolving patient and family values. A sustainable FP support system requires multidisciplinary collaboration.

Support challenges for HCTCs in facilities without in-house FP systems:

-Establishing smooth coordination with nearby reproductive clinics

-Building a continuous support framework from diagnosis through long-term follow-up (LTFU)

-Securing opportunities for shared reflection based on patients' personal values

-Strengthening multidisciplinary collaboration among transplant physicians, reproductive specialists, psychosocial support staff, and LTFU providers

Poster Presentation

Allied Health including Nursing, Pharmacology, and Laboratory Aspects

SINGLE-CENTER HEMATOPOIETIC STEM CELL COLLECTION FROM SEVERE THALASSEMIA PATIENTS FOR GENE THERAPY: 42 CASES

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Aims:
The target $CD34^+$ cell dose of gene therapy should achieve≥10×10^6/kg.However, abnormal RBCs in thalassemia may reduce CD34+ collection efficiency due to compromise apheresis layer. This single-center study evaluates the safety and efficacy of peripheral blood stem cell (PBSC) collection in severe thalassemia patients undergoing gene therapy.

Methods:
Between January 2023 and April 2025, a total of 42 cases with severe thalassemia (26 males, 16 females; age range 5-30 years; including 3 splenectomized cases) underwent PBSC collection for gene therapy in our single-center. PBSC collections were performed using three apheresis devices: Spectra Optia (n=29), Spectra COBE (n=9), and COMTEC (n=4), following standardized operating procedures. all patients received stem cell mobilization with G-CSF (10 μg/kg/day for 5 days) combined with plerixafor (20 mg,11 hours pre-apheresis).RBC priming were performed for 6 patients weighing <30 kg and 3 patients with hemoglobin <80 g/L. Femoral vein catheterization was performed in 11 cases, while peripheral venous access was performed in 31 cases. The procedure parameters were individualized based on CD34+ cell counts and patient tolerance: circulating blood volume 6,231–19,000 mL (2.92–5.86×total blood volume), flow rate 21.2–55 mL/min, ratio of citrate anticoagulant 1:10–1:15 .Continuous color monitoring of collection product with appropriate adjustments were made to achieve the best collection effect.

Results:
PBSC collection was successfully completed in 42 cases, with 7 cases (16.66%) experiencing adverse reactions.Adverse reactions included mild peripheral paresthesia (5 cases) and dizziness (2 cases), all relieved after calcium supplementation. Collection volume 139-440 mL, MNCs yield 8.29×10^8/kg to 48.17×10^8/kg , $CD34^+$ cells yield 2.10×10^6/kg to 69.28×10^6/kg, Hct 1.9%-11.2%. 40 cases (95.2%) achieved target $CD34^+$ cell yields (>10×10^6/kg) in a single collection,

with collection efficiencies ranging from 31% to 83%.However, 2 splenectomized cases required consecutive two collections to reach target doses, demonstrating markedly lower efficiencies (6%-13%). All collected cell products met gene therapy preparation standards.

Conclusion:

This single-center study demonstrated safety and efficacy of PBSC collection from severe thalassemia patients undergoing gene therapy. This approach was feasible across all age groups and provided a reliable cell source for gene therapy, including in splenectomized patients.