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ABSTRACT BOOK

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No child should die of cancer: cure for more, care for all

The development of this Abstract Book was led by Julia Georgieva, SIOP Programme Coordinator and Tzvetomira Laub, SIOP Chief Programs Officer.

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Table of Contents

01 Haematological Malignancies — Oral Presentation	**4**
02 Haematological Malignancies — Poster	**4**
03 Ocular Oncology — Poster	**34**
04 Neuro-Oncology — Oral Presentation	**35**
05 Neuro-Oncology — Poster	**37**
06 Solid Tumors — Oral Presentation	**44**
07 Solid Tumors — Poster	**49**
08 Implementation Science — Oral Presentation	**76**
09 Implementation Science — Poster	**81**
10 Nursing — Oral Presentation	**91**
11 Nursing — Poster	**93**
12 Other — Oral Presentation	**93**
13 Other — Poster	**95**
Index by Cancer Type & Topic	**100**
Index by Author	**103**

01. HAEMATOLOGICAL MALIGNANCIES — ORAL PRESENTATION

Abstract 101

Mini-ATG/TBI Conditioning Regimen Facilitates Cure of Acquired Aplastic Anemia in Children via Unrelated Umbilical Cord Blood Transplantation

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Purpose: Umbilical cord blood transplantation (UCBT) represents a alternative therapeutic option for patients with acquired aplastic anemia (AA) lacking a HLA-matched sibling donor. However, high incidences of graft failure and acute graft-versus-host disease (aGVHD) following UCBT remain major challenges. The optimal conditioning regimen for UCBT in AA is yet to be established.

Methods: We enrolled patients with newly diagnosed AA or those refractory to prior Immunosuppressive therapy. This single-center prospective UCBT trial (registered at www.chictr.org.cn #ChiCTR2400088868), initiated in March 2024, compared outcomes using a mini-ATG/TBI-based regimen against historical data from our center prior to March 2024. The mini-ATG/TBI regimen comprised: rabbit ATG (rATG, 2.5 mg/kg) on day -7, total body irradiation (TBI, 3 Gy) on day -7, fludarabine (FLU, 40 mg/m²) on days -6 to -2, and cyclophosphamide (CTX, 40 mg/m²) on days -4 and -2. The historical regimen consisted of TBI (4 Gy) on day -7, FLU (40 mg/m²) on days -6 to -2, and CTX (60 mg/m²) on days -3 and -2. Outcomes evaluated included aGVHD rates, graft failure rate, engraftment rate, time to neutrophil engraftment, time to platelet engraftment, and overall survival.

Results: 1. A total of 27 patients were enrolled: 18 in the mini-ATG/TBI group and 9 in the conventional group. The cohort included 13 males and 14 females, with a median age of 7 years (range: 2–15 years). No significant age difference existed between groups (P=0.665). 2. aGVHD: Grade III-IV aGVHD occurred in 2/18 patients (11.11%) and Grade I-II in 5/18 in the mini-ATG/TBI group, compared to 4/9 (44.44%) and 3/9 in the conventional group. The incidence of severe (Grade III-IV) aGVHD was significantly lower in the mini-ATG/TBI group (11.11% vs. 44.44%, P=0.05). 3. Engraftment: One patient in the mini-ATG/TBI group experienced graft failure due to early EBV viremia (within 1month post-transplant) complicated by suspected splenic PTLD and secondary hemophagocytic lymphohistiocytosis (HLH). This patient achieved disease-free survival after salvage haploidentical transplantation using an HLH-directed conditioning regimen. Engraftment was successful in the remaining 26 patients. Engraftment rates of the two groups were 94.12% vs. 100% (P=0.471). Median donor chimerism on day +7 was 71.62% (range: 21.84%–92.75%), with no significant difference between groups (72.25% vs. 63%, P=0.522). Complete donor chimerism (≥99%) was achieved by day +14 in all evaluable patients. Median time to neutrophil engraftment was 15 days (range: 12– 30) in the mini-ATG/TBI group and 16 days (range: 13–27) in the conventional group (P=0.287). Median time to platelet engraftment was 24 days (range: 16–67) vs. 24 days (range: 18–36) (P=0.427). In the mini-ATG/TBI group, odd-numbered patients received avatrombopag starting on day +7 to promote platelet engraftment, while even-numbered patients received thrombopoietin (TPO). Subgroup analysis showed no significant difference in platelet engraftment time (23 days, range: 20-33 days vs. 25.5 days, range: 16-67 days; P=0.382). 4.Transplant Related Complications: Rates of sepsis, transplant-associated thrombotic microangiopathy (TA-TMA) and EBV infection did not differ significantly between two groups. However, CMV viremia incidence differed significantly (6.25% vs. 66.67%, P=0.001), attributed to the widespread use of letermovir for CMV prophylaxis after 2024. Hemorrhagic cystitis also has significantly difference (0% vs. 33.33%,P=0.011). 5.Survival: The estimated 2-year overall survival (OS) rate was 92.86% (±6.88%) for the mini-ATG/TBI group and 88.89% (±10.48%) for the conventional group, showing no statistically significant difference (χ^2=0.001, P=0.971).

Conclusion: These data support the mini-ATG/TBI regimen as an effective conditioning approach for UCBT in acquired aplastic anemia, demonstrating particular utility in pediatric patients or low body weight adults.

Abstract 102

Therapy acquired genomic and MMR signatures and clonal evolution patterns define up to one-third of pediatric B-cell acute lymphoblastic leukaemia relapses: Integrated analysis with clinical 6-MP dosing

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Purpose: Acquired thiopurine resistance and mismatch repair (MMR) defects are implicated in pediatric B-cell acute lymphoblastic leukemia (B-ALL) relapse. However, there is paucity of real-world genomic and clinical data o this subject.

Methods: Pediatric B-ALL relapse cases (2019-2024) were primarily subjected to whole exome sequencing (250x depth; n=70) and partly deep targeted sequencing (>1000x;n=5). We derived per-sample frequencies of resistance genes, single base mutational signatures, TMB-High (≥10/Mb), MSI-H, and TPMT/NUDT15 status. Clinical variables included median weeks off 6-MP therapy and median 6-MP dose.

Results: A total of 18/75 (24%) relapse cases showed SBS87 (Thiopurine related), SBS6/15 (MMR related) or Thio-dMMR mutational signature. Thiopurine resistance clones in either NT5C2 (13), PRPS1 (2) or TP53 (7) genes were noted in 19/75 (25%) cases while germline or somatic clones in MMR genes of MSH6 (4), MSH2 (3), PMS2 (7), MLH1(1), PMS1 (4), MSH3 (1), MSH4 (2) were noted in 19/75 (25%). Thiopurine gene resistance and or MMR gene mutations overlapped with either SBS87/6/15/Thio-dMMR signature in 55.5% (10/18) cases. The median weeks off 6-MP therapy was 4 (IQR 2–8); median 6-MP dose 54 (47–60); while TPMT/NUDT15 SNPs were noted in 11/75 (14.6%) cases. TMB-High (>10 mut/Mb) was noted in 10% (7/70) cases while MSI-High was noted in 11% (8/70). TP53 & MSH6 were significantly enriched for TMB-H (OR 15.8, p = 0.046). Directional clinical associations revealed modestly lower 6-MP mean dose to be related with NT5C2 resistant mutations and or SBS87 signature.

Conclusion: This real-world data highlights a bidirectional interplay between thiopurine dosing pattern, evolution of resistant mutation and clonal selection including a sub-set predisposed to hypermutations due to MMR aberrations. Hence, there is a need for an integrated clone–signature–dose profiling strategy to guide dosing and genomic surveillance to mitigate relapse risk in pediatric B-ALL cases.

Abstract 103

Belumosudil in pediatric patients with steroid-refractory chronic graft-versus-host disease

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Purpose: Belumosudil has been introduced to treat steroid-refractory chronic graft-versus-host disease (SR-cGVHD) in adult patients. Similarly, cGVHD occurred in nearly 25% of pediatric allogeneic hematopoietic cell transplant (HCT) recipients. But the lack of data in pediatric SR-cGVHD patients contributes to poor prognosis, especially in young children aged<12. We aim to investigate the efficacy and safety of belumosudil in pediatric patients after failing multiple lines of treatment (LOTs) as salvage therapy.

Methods: This study included 49 HCT pediatric patients with SR-cGVHD failing multiple LOTs, and all patients received belumosudil. The primary endpoint was the overall response rate (ORR), duration of response (DOR), cumulative response rate, response rate by organs, failure-free survival (FFS), and overall survival (OS), and adverse effects (AEs) during belumosudil administration were documented.

Results: In all enrolled patients, 37 patients were <12 years old, the median age was 9 years (range, 1-16), and 33 patients were male. The median time of

follow-up was 11 (6-18) months. According to the 2014 NIH Consensus Criteria, 18 patients had severe cGVHD at screening, and 30.6% (15/49) had involvement of ≥3 organs. The median time to response was 6 weeks (range, 2-16), and the ORR was 81.6% (61.1% in the severe cGVHD group and 93.5% in the moderate group). The organ-specific analyses demonstrated an ORR of 93.1% in the skin, 90.0% in the mouth, 90.9% in the liver, 94.4% in the gastrointestinal tract and 71.4% in the lung. Patients aged<12 years with persistent cGVHD manifestations received belumosudil with an ORR of 81.1%. The estimated DOR rate at 60 weeks was 74.1% (95% CI: 66.5%-81.7%) and the cumulative response rate was 87.6%. The overall FFS was 77.0% (95% CI, 70.1-83.9%) and none of the patients died during follow-up. Cytopenias and infection were documented in five patients during belumosudil treatment, and none of them was of grades 3-4. Thus, the AE rates were low in patients with belumosudil.

Conclusion: This is one of the earliest studies to describe the role of belumosudil for SR-cGVHD in the pediatric population. Overall, belumosudil was effective and well-tolerated in pediatric patients with SR-cGVHD and the response was rapid. A clinically meaningful improvement of cGVHD was found in younger children aged<12 years, given the off-label indication in this age class. Further prospective studies with a larger sample size may be warranted to verify the results in the pediatric population.

Abstract 104

Blinatumomab for clearance of next-generation sequencing measurable residual disease in pediatric B-cell acute lymphoblastic leukemia: a single-center observational study

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Purpose: To investigate the efficacy of blinatumomab in eliminating next-generation sequencing measurable residual disease (NGS-MRD) in pediatric patients with B-cell acute lymphoblastic leukemia (B-ALL).

Methods: This single-center retrospective study enrolled pediatric B-ALL patients with pretreatment positive NGS-MRD who received blinatumomab for more than 7 days at our hospital from June 2020 to December 2025. Bone marrow samples were collected before and after treatment for NGS-MRD detection. The clearance rates of different immunoglobulin chains were calculated, the correlation between clonal clearance and clinical prognosis was analyzed, and treatment-related adverse events were systematically recorded.

Results: A total of 122 eligible patients were enrolled, including 16 cases of relapsed/refractory B-ALL, 37 cases with persistent MRD positivity, 27 high-risk B-ALL cases receiving intensive treatment, and 42 cases with other indications. Among them, only 13 patients had concurrent positive flow cytometry (FCM)-MRD results. Before treatment, 332 primary clones and 90 novel clones were identified, with IGH being the predominant subtype (34.4%). For primary clones, the clearance rates of IGH, IGH-DJ, IGK, IGKDE and IGL were 71.5%, 54.7%, 65.0%, 64.6% and 71.4%, respectively; the corresponding clearance rates for novel clones were 60.0%, 20.6%, 38.9%, 37.5% and 100.0%, respectively. Patients with cleared primary IGH clones had a significantly higher 5-year event-free survival rate (85.5% vs 66.9%, P=0.0042), while the clearance status of other immunoglobulin chains and novel clones showed no significant correlation with prognosis. 59.0% (72/122) of patients experienced grade ≥3 adverse events, mainly including hematological toxicity (63 cases) and infection (10 cases). All adverse events were resolved after symptomatic supportive treatment, and no treatment-related deaths occurred.

Conclusion: Blinatumomab yields favorable efficacy in clearing NGS-MRD in pediatric B-ALL patients with manageable safety profile, and it can serve as a valid therapeutic choice for pediatric B-ALL patients with positive NGS-MRD.

Abstract 105

REAL-WORLD OUTCOMES OF IN-HOUSE ANTI-CD19 CAR-T THERAPY FOR PEDIATRIC R/R B-ALL IN BELARUS

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Purpose: Chimeric antigen receptor T-cell (CAR-T) therapy is an effective treatment for relapsed/refractory B-cell acute lymphoblastic leukemia (R/R B-ALL). We report single-center outcomes of anti-CD19 CAR-T therapy using locally manufactured products.

Methods: From 2020 to 2026, 20 patients with R/R B-ALL received anti-CD19 CAR-T therapy at the Belarusian Research Center for Pediatric Oncology, Hematology, and Immunology. Median age at infusion was 12.5 years (range 3.7–30.3). Patients had received a median of 2 prior treatment lines (range 1–5). At infusion, 12 patients had active disease, 4 were MRD-positive, and 4 MRD-negative. CAR-T cells containing an mFMC63 scFv, 4-1BB co-stimulatory domain, and CD3ζ signaling domain were produced from leukapheresis-derived CD4+ and CD8+ T cells, expanded separately, and infused in an approximately 1:1 ratio after lymphodepletion. Median dose was 0.8×10^6 cells/kg.

Results: Peak CAR-T expansion occurred on day 10 (median 37.8 cells/μL). Three patients died before response evaluation. Among evaluable patients (n=17), the overall response rate was 88.2%, with MRD-negative remission in 80% of responders. Eight responders underwent subsequent allogeneic HSCT. Grade ≥3 cytokine release syndrome occurred in 25% of patients (one fatal), and grade ≥3 ICANS in 25% (three fatal). Early ICAHT was observed in 55%, and severe infections in 40% (two fatal). Toxicity was not associated with tumor burden, prior therapies, or CD4/CD8 ratio. One-year overall survival was significantly higher in patients infused in remission compared with active disease (75% vs 37%, p=0.042). Four-year OS was 42.5%, and 3-year event-free survival 28.7%.

Conclusion: Anti-CD19 CAR-T therapy achieves high remission rates in R/R B-ALL and frequently serves as a bridge to allo-HSCT. Despite significant toxicity, outcomes are comparable to real-world data and support its role in improving survival.

Abstract 106

Development and Multicenter Validation of Serial Biomarker-Based Machine Learning Models for the Diagnosis of GVHD After Pediatric Allogeneic Hematopoietic Stem Cell Transplantation

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Purpose: Acute graft-versus-host disease (aGVHD), especially severe aGVHD, remains a major complication after pediatric allogeneic hematopoietic stem cell transplantation. However, real-time diagnosis is difficult because post-transplant manifestations often overlap with infection and other complications. We aimed to develop and validate machine-learning models based on serial biomarker monitoring to assist the contemporaneous diagnosis of GVHD.

Methods: A multicenter retrospective study was conducted in pediatric allo-HSCT recipients within Day 0-100 after transplantation. Six biomarkers (IL-6, IL-8, TNFR1, ST2, Reg3α, and Elafin) and time from transplantation were used as predictors. Each measurement was treated as an index record and aligned with clinically adjudicated GVHD onset. Suspected events were classified using a MAGIC-based diagnostic certainty framework, with confirmed and probable events considered positive in the primary analysis. Separate models were developed for GVHD and severe GVHD (sGVHD; grade III-IV aGVHD). Data from our center were used for model development and internal validation/recalibration, and a merged cohort from two external centers was used for independent external validation. Candidate algorithms were compared using patient-level resampling, and performance was assessed in terms of discrimination, calibration, and clinical utility.

Results: The development validation cohort included 216 patients (1,859/2,085 measurements for the GVHD/sGVHD models), and the external validation cohort included 88 patients with 106 symptom-triggered measurements. In external validation, the GVHD model achieved an AUROC of 0.781 (95% CI, 0.685-0.869) and an AUPRC of 0.805 (95% CI, 0.697-0.894), while the sGVHD model achieved an AUROC of 0.806 (95% CI, 0.710-0.898) and an AUPRC of 0.733 (95% CI, 0.583-0.850). After internal recalibration, both models showed acceptable calibration in the external cohort and demonstrated net clinical benefit across clinically relevant threshold probabilities on decision-curve analysis.

Conclusion: Serial biomarker-based machine-learning models showed promising performance for the contemporaneous diagnosis of GVHD after pediatric

allo-HSCT. These findings support the potential role of dynamic biomarker monitoring as an adjunctive tool for real-time diagnostic support and risk-informed clinical decision-making.

Abstract 107

Effect of Vaccination Timing (3 Months vs 6 Months Post-Chemotherapy) on Protective Antibody Titre Development in Children with Acute Lymphoblastic Leukemia: A Randomized Controlled Trial.

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Purpose: Children receiving chemotherapy for acute lymphoblastic leukemia (ALL) are immunosuppressed, resulting in partial or complete loss of protective immunity against vaccine preventable diseases (VPD). Re-vaccination is carried out after completion of therapy (ACT), with guidelines varying regarding appropriate timing of vaccination. Literature assessing effectiveness of prior vaccination and the optimal efficacy and timing of re-vaccination is scant, with guidelines mentioning initiation of revaccination at either 3- or 6-months ACT. We aimed to compare antibody development against various VPDs after re-vaccination at 3-months vs 6-months in children treated for ALL.

Methods: A prospective randomized comparison was done (Jan 2021-June 2022). Fifteen controls (newly diagnosed ALL patients) were assessed for protective antibody titer. Thirty-nine children: 20 were vaccinated at 3-months and 19 at 6-months ACT.

Results: The median age was- controls (n=15): 6.3 years; cases (n=39): 6.5 years. Antibody titer at diagnosis of ALL was adequate against rubella in 100%, diphtheria:94%, hepatitis B:87%; and inadequate against measles:53%, and mumps:34%. The protective antibodies had decreased by end of therapy, with protection against Rubella present in 67%, diphtheria:46%, hepatitis B:26%, and measles:18%. Single dose of vaccination carried out at 3-months versus 6-months after completion of therapy generated equal protective antibody levels against rubella in 90% vs 95% children; diphtheria in 95% vs 95%; mumps in 85% vs.79%; hepatitis B in 75% vs.70%, respectively. Antibody against measles were more effective at 3 months than at 6 months in 65% vs. 32%, p= 0.02. Single dose of vaccine was inadequate for generating antibody levels against measles and hepatitis B in 50% and 54% children.

Conclusion: Vaccination at 3 months after completion of therapy provides equal efficacy against VPDs as compared to 6 months in children who have completed therapy for ALL, providing earlier protection. Single dose of mumps and hepatitis B is insufficient, and repeat dosing is required.

Abstract 108

Comparison of Long-Term Efficacy Between 14-Day and 28-Day Courses of Blinatumomab in Pediatric B-Cell Acute Lymphoblastic Leukemia Using Different MRD Assessment Methods

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Purpose: In patients with B-cell acute lymphoblastic leukemia (B-ALL), blinatumomab effectively eliminates minimal residual disease (MRD) and improves prognosis. Next-generation sequencing (NGS) enables more sensitive MRD detection by identifying specific immunoglobulin heavy chain (IgH) rearrangements. This study investigated the correlation between 14-day (14D) and 28-day (28D) blinatumomab treatment plans and MRD clearance rates assessed by different detection methods, as well as their impact on prognosis.

Methods: A retrospective analysis was conducted on 29 B-ALL patients, including 19 in the 14D group and 10 in the 28D group. All patients were in first complete remission (CR1) and received one course of blinatumomab treatment (either 14-day or 28-day) during their therapy for various reasons.

Results: After one course of blinatumomab, 18 of 27 evaluable patients achieved complete clearance of NGS-MRD. Overall, 23 patients achieved NGS-MRD negativity, with clearance rates of 77.8% in the 14D group and 75% in the 28D group. No statistically significant difference in NGS-MRD clearance was observed between the two groups (P=0.683). The concordance between NGS-MRD and multiparameter flow cytometry MRD (MFC-MRD) based on positive/negative results was 74.07% (P=0.000), while the concordance based on detectability was 57.41% (P=0.018). By the follow-up cutoff date, 6 patients had relapsed, including 2 who died (both in the 14D group). The 3-year overall survival (OS) rates were 85.3% ± 0.101 for the 14D group and 100% for the 28D group (P=0.336). The 3-year event-free survival (EFS) rates were 73.7% ± 0.185 and 90.0% ± 0.095 for the 14D and 28D groups, respectively (P=0.485).

Conclusion: The 14D and 28D blinatumomab regimens demonstrated comparable efficacy in MRD clearance, both superior to chemotherapy alone. NGS-MRD detection exhibited higher sensitivity. The two deaths occurred in the 14D group, both in high-risk patients who did not subsequently undergo transplantation. This suggests that for patients with relapsed/refractory disease, transplantation or novel therapeutic approaches remain necessary to improve outcomes. When economically feasible, the 28D blinatumomab regimen is still recommended to potentially enhance MRD clearance.

Abstract 109

Improving Outcomes of Hematopoietic stem cell transplantation in Transfusion dependent Thalassemia: Lessons from two decades of experience

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Purpose: Objective: To determine long term outcomes of allogenic bone marrow transplant in transfusion dependent thalassemia, identify factors that influence survival and common complications in resource limited setting.

Methods: Study design: Descriptive study conducted in armed forces bone marrow transplant center Pakistan from Oct 2001 to Dec 2023.Patient demographics, pre-transplant status, stem cell source, conditioning regimen and post-transplant outcomes were recorded.

Results: A total of 411 transplants were done, mean age of diagnosis was 10.80± 12.76 months and mean age at BMT was 6.0±3.1 years. There were 265 male patients and 145 females. 91 % patients had irregular chelation, 39 % patients had ferritin between 1000-2000 ng/dl, 55.4% had < 50 red cell transfusions, 14.40% were HCV +ve and 91.70% were CMV +ve. 259(63%) patients belonged to Pesaro risk group III. 375 (92%) donors were fully HLA matched siblings. Bu14Cy200ATG/TT conditioning protocol was used in 24.8% patients and FluBu14Cy160 conditioning was used in 75.2% patients. Bone marrow was the source of stem cells in 337 (82%) patients with median TNC 5.11±1.32 x 108/kg, median CD34 7.66±9.20 x 106/kg and median CD3 4.54±2.86 x 107/kg. Neutrophils and platelets engrafted on a median of day+ 14.56±3.63 and day+ 28.04±12.84 respectively. Neutropenic fever (83.4%), hypertension (79.6%), Mucositis/gut toxicity (49.2%), aGVHD (37.9%) and VOD (14.3%) were common complications. At a median follow up of 24 years, overall survival (OS) was 79.1% and disease-free survival was 68.1%. In univariate analysis, age more than 10 years (p<0.001), liver more than 5 cm below RCM (p<0.001), Pesaro grade IV (p<0.000) and VOD (p<0.001) significantly affected overall survival and disease-free survival. 15.3% patients had primary graft failure. Treatment related mortality occurred in 86 (20.92%) patients, infections (58%)- GVHD (14%) and VOD (11%) being common causes.

Conclusion: Good infection control, younger age and less blood transfusions before transplant, lower dose of ATG (5 mg/kg) and conditioning Flu120Bu16Cy160TG4.5-7.5 can improve overall survival.

Abstract 110

Fever and Otorrhea at Onset Are Independent Risk Factors for Poor Response to CCHG-LCH-2019 Regimen in Pediatric Langerhans Cell Histiocytosis: A 10-Year Single-Center Retrospective Study

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Purpose: Despite significant improvements in survival rates for Langerhans cell histiocytosis (LCH) through global collaborative efforts, the recurrence rate

remains high.The purpose of our study was to analyze the clinical characteristics and treatment outcomes of patients at our center over the past decade.

Methods: We retrospectively collected data from LCH patients diagnosed and treated at the Children's Medical Center, Sun Yat-sen Memorial Hospital, Sun Yat-sen University between May 1, 2015, and October 31, 2025. Comparative analyses were performed regarding clinical characteristics, BRAF V600E mutation, inflammatory cytokines, and treatment responses.

Results: A total of 105 patients were included, with an overall survival (OS) rate was 100%. The 1-year progression-free survival (PFS) rate was (79.0± 4.0) %, and the 3-year and 5-year PFS rates were both (72.8 ± 4.4) %. From May 1, 2015, to November 30, 2018, 35 patients were treated with the JLSG-96 regimen. Among whom 6 (17.1%) exhibited a poor response. The 1-year PFS was (88.6± 5.4) %, and the 3-year and 5-year PFS rates were both (82.9±6.4) %. No statistically significant differences were observed in baseline clinical characteristics or inflammatory cytokines between the poor-response and good-response groups. From December 1, 2018, to October 31, 2025, 70 patients received the CCHG LCH 2019 regimen, with 22 (31.4%) showing a poor response. The 1 year PFS rate was (74.2 ± 5.2)%, and the 3 year and 5 year PFS rates were both (67.7 ± 5.7)%. Univariate analysis revealed that fever at onset, otorrhea, positive bone marrow BRAF V600E mutation, and elevated peripheral blood levels of sIL-2R, IL-6, and TNF-α were positively correlated with poor treatment response, while local pain was negatively correlated. Multivariate Cox proportional hazards regression analysis identified fever (HR=7.34, P<0.001) and otorrhea (HR=5.26, P=0.001) at onset as independent risk factors for poor response to the CCHG-LCH-2019 regimen. In patients with poor response to first-line therapy, disease was well controlled after salvage therapy with MAPK pathway inhibitors or cladribine combined with cytarabine.

Conclusion: Pediatric LCH patients have excellent overall survival. The JLSG 96 regimen was associated with higher 1 year, 3 year, and 5 year PFS rates than the CCHG LCH 2019 regimen. Salvage therapy achieved favorable disease control in patients with poor response to first line treatment. Fever and otorrhea at onset are independent risk factors for poor response to the CCHG LCH 2019 regimen, suggesting that more intensive upfront therapy may be warranted in these patients.

Abstract 111

Elevated IL-6 and B-Cell Dysregulation Predict Poor Response to First-Line Therapy in Pediatric Langerhans Cell Histiocytosis: Otorrhea as a Clinical Sentinel of Immune-Mediated Treatment Resistance

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Purpose: This study aimed to identify high-risk factors associated with poor response to first-line therapy in pediatric patients with Langerhans cell histiocytosis (LCH), with a specific focus on the correlations among clinical characteristics, immunological biomarkers, and therapeutic outcomes.

Methods: We retrospectively analyzed pediatric patients with LCH treated between December 2018 and March 2024. Participants were stratified into two cohorts based on their response to first-line treatment: favorable responders (FRG) and poor responders (PRG). Comparative analyses of clinical characteristics and peripheral immune profiles were performed between the two groups to identify potential immunological predictors of the therapeutic efficacy. These findings are anticipated to inform individualized treatment strategies and facilitate the discovery of novel biomarkers for the management of LCH.

Results: Among the 54 enrolled patients, 23 (42.6%) were classified as PRG. Clinically, PRG exhibited significantly higher incidences of otorrhea (P = 0.009) and liver involvement (P = 0.049), accompanied by a trend toward increased bone marrow BRAF-V600E mutation (P = 0.055), a genotype closely linked to treatment resistance in LCH. Immunologically, PRG demonstrated elevated serum levels of soluble interleukin-2 receptor (sIL-2R; P = 0.044), interleukin-6 (IL-6; P = 0.008), immunoglobulin G (IgG; P < 0.001), and erythrocyte sedimentation rate (ESR; P = 0.01). Additionally, PRG presented with an altered lymphocyte subset profile, characterized by an increased CD20 B-cell/lymphocyte (LY) ratio (P = 0.007) and total B-cell count (P = 0.01), whereas total T-cell counts (P = 0.037), CD8 T-cell counts (P = 0.02), and Th17 cell proportions (P = 0.011) were significantly decreased. Predictive analysis revealed that several clinical and immunological parameters were associated with treatment response, including otorrhea (AUC = 0.663), IL-6 (AUC = 0.685), IgG (AUC = 0.750), ESR (AUC = 0.685), total B-cell count (AUC = 0.661), Th17 cell proportion (AUC = 0.675), and CD20 B-cell/lymphocyte ratio (AUC = 0.671). Correlation analysis further demonstrated that otorrhea was moderately positively associated with IL-6 (r = 0.57) and total B-cell count (r = 0.54), and inversely correlated with Th17 cell proportion (r= -0.56). These results suggest a potential interplay between the clinical manifestations and immune dysregulation in LCH progression.

Conclusion: Elevated IL-6, IgG, and ESR levels, along with aberrant B-cell- and T-cell subset profiles, were significant predictors of poor response to first-line treatment in pediatric LCH. Notably, higher IL-6 levels were associated with diminished therapeutic efficacy, as reflected by the increased incidence of otorrhea. More aggressive treatment strategies may be warranted for pediatric patients with LCH presenting with otorrhea at disease onset. These findings underscore the clinical value of early immune profiling for risk stratification and position IL-6 and B cell dysregulation as promising targets for adjunctive therapy in high-risk LCH cases. To our knowledge, this is the first study to systematically link B cell subset dysregulation with otorrhea and treatment response in pediatric LCH.

Abstract 112

Clinical Study of HAG Regimen in Induction Chemotherapy for Pediatric Acute Myeloid Leukemia

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Purpose: To investigate the clinical efficacy and safety of the HAG regimen (low-dose homoharringtonine + cytarabine + granulocyte colony-stimulating factor) in induction chemotherapy for pediatric acute myeloid leukemia (non-M3 subtype), and to provide evidence-based references for the selection of clinical treatment regimens for pediatric AML.

Methods: A retrospective,analysis was conducted on the clinical data of 184 newly diagnosed pediatric patients with acute myeloid leukemia (non-M3 subtype) admitted from June 2019 to January 2025. The patients were divided into the HAG group (treated with the HAG regimen) and the non-HAG group (treated with anthracycline combined with cytarabine-based regimen) according to the induction chemotherapy regimen. SPSS 31.0 software was used for statistical analysis. The chi-square test was adopted to compare the efficacy and safety indicators between the two groups, and the Kaplan-Meier method was used to plot survival curves and analyze the differences in survival rates.

Results: The overall complete remission (CR) rate in the HAG group was significantly higher than that in the non-HAG group (89.3% vs 57.6%, P 0.001), and the minimal residual disease (MRD)-negative CR rate was also significantly higher (44.7% vs 22.2%, P=0.003). Stratified analysis showed that the CR rates in the HAG group were significantly superior to those in the non-HAG group among intermediate-risk and high-risk patients (98.2% vs 78.9%, P=0.003; 82.2% vs 40.0%, P 0.001). In patients with poor prognostic genes such as NUP98-NSD1 and MLL rearrangement, the CR rates in the HAG group tended to be higher (70.0% vs 42.9%, P=0.263; 73.3% vs 50.0%, P=0.257). In terms of safety, the incidence of grade 3-4 anemia (36.4% vs 73.1%, P 0.001) and the incidence of aspergillus pneumonia (12.1% vs 32.7%, P=0.001) in the HAG group were significantly lower than those in the non-HAG group. Survival analysis revealed that the 1-year, 3-year and 5-year overall survival rates (93.5%, 88.7%, 84.0% vs 87.6%, 73.2%, 73.2%, P=0.081) and event-free survival rates (86.3%, 74.8%, 62.4% vs 80.1%, 65.2%, 60.8%, P=0.333) in the HAG group were slightly higher than those in the non-HAG group

Conclusion: The HAG regimen exerts remarkable efficacy in induction chemotherapy for pediatric acute myeloid leukemia, with particularly prominent advantages in intermediate-risk and high-risk patients. Compared with the traditional high-intensity chemotherapy regimen , it has lower hematological toxicity and fewer infection-related complications, presenting higher safety. It can be used as one of the preferred regimens for induction chemotherapy in pediatric

acute myeloid leukemia.

02. HAEMATOLOGICAL MALIGNANCIES — POSTER

Abstract 113

HYPERSENSITIVITY REACTIONS TO ASPARAGINASE THERAPY IN PEDIATRIC ACUTE LYMPHOBLASTIC LEUKEMIA AND LYMPHOBLASTIC LYMPHOMA PATIENTS AT CHILDREN HOSPITAL No2, VIETNAM

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Purpose: Asparaginase can cause adverse reactions, especially hypersensitivity reactions (HSR), leading some pediatric patients with acute lymphoblastic leukemia and lymphoblastic lymphoma to discontinue the medication. This study evaluates the frequency of HSR with L-asparaginase (L'ASP) and the risk factors for severe HSR.

Methods: A retrospective study in 138 cases at Children's Hospital No2 from 2023 to 2025.

Results: There were 49 HSR recorded in 42 patients (30.4%). HSR severity was grade 1 (21.4%), grade 2 (50%), grade 3 (16.7%), grade 4 (11.9%). Adverse reaction in unique course was noted in 85.7% of patients and the first HSR happened commonly in consolidation (52.4%). HSR grade 3/4 prevalently occurred from the 2nd to the 4th dose of L'ASP the course (41.7%). Forty-one percentage of patients experienced grade 1,2 HSR before developing grade 3 or 4 allergies. Prophylactic corticosteroids and extending time of L'ASP infusion were recorded in 71% of patients with drugs re-admistration. Patients with initial HSR grade 1 got success with reinfusion in 88.9% suggested non-antibody-mediated infusion reactions may occur in this group while just 61% of patients with history of HSR grade 2,3 overcame recurrent allergies. Only 5 out of 17 cases for whom Erwinia asparaginase was indicated received treatment. Previous grade 1,2 HSR is considered as risk factor for more serious allergies later (OR 11.2, $p< 0.05$).

Conclusion: HSR rate remains high, coupled with a low rate of Erwinia asparaginase switching. Further testing is needed to differentiate between HSR and infusion reactions, as well as to quantify ASP activity levels.

Abstract 114

FATAL CONGENITAL MULTISYSTEM LANGERHANS CELL HISTIOCYTOSIS IN A PRETERM NEONATE: A CASE REPORT

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Purpose: Langerhans cell histiocytosis (LCH) is a rare disorder, with an estimated incidence of 1–2 cases per million neonates, characterized by clonal proliferation of abnormal Langerhans cells. The clinical spectrum ranges from localized single-organ involvement to disseminated multisystem disease. In fetal and neonatal cases, LCH is broadly categorized into skin-limited disease and multisystem involvement. The latter, particularly when involving the liver, spleen, and hematopoietic system, is associated with a high risk of mortality. We report a case of congenital multisystem LCH diagnosed at autopsy in a preterm neonate.

Methods: A complete autopsy was performed, including gross examination, histopathological evaluation, and immunohistochemical analysis. The findings were compared with previously reported cases in the literature.

Results: A preterm neonate (36+2 weeks of gestation) died on the sixth day of life after receiving supportive care. Autopsy revealed abdominal distension and multiple cutaneous lesions measuring 0.5–1.2 cm in diameter, presenting as papules and vesicles with a hemorrhagic appearance. Internal examination demonstrated hepatomegaly (230.3 g), splenomegaly (28.5 g), thymic enlargement (12.5 g), and generalized lymphadenopathy (up to 1.5 cm). Histopathological analysis showed widespread infiltration of the skin, lungs, epicardium, liver, spleen, gastric mucosa, adrenal glands, and kidneys by large round to oval cells with abundant eosinophilic cytoplasm and characteristic nuclear grooves ("coffee-bean" appearance), along with occasional multinucleated giant histiocytes and focal necrosis. Immunohistochemistry revealed positivity for S100 and CD45, while CD3 and CD20 were negative. The Ki-67 proliferation index exceeded 40%.

Conclusion: Congenital LCH should be considered in neonates presenting with hemorrhagic skin lesions. Early skin biopsy is recommended for prompt diagnosis. Multisystem congenital LCH carries a high risk of fatal outcome; therefore, early histopathological confirmation and initiation of targeted therapy are crucial to improve survival.

Abstract 115

Poor Outcomes Of FLT3-Mutated Pediatric Acute Myeloid Leukemia In A Resource-Constrained Setting: Urgent Need For Targeted Therapeutic Strategies

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Purpose: FLT3-mutated acute myeloid leukemia (AML) is a well-recognized high-risk subtype, yet real-world outcomes from low- and middle-income countries remain underreported. Limited access to targeted therapies and transplant may further exacerbate poor prognosis. We present outcome data highlighting critical gaps in care.

Methods: We performed a retrospective cohort study of pediatric AML patients diagnosed between 2022–2025 at a tertiary cancer center in India. Patients harboring FLT3 mutations (ITD/TKD) were analyzed for baseline characteristics, co-mutations, treatment response, and survival outcomes. Event-free survival (EFS) was defined as relapse, refractory disease, or death.

Results: FLT3 mutations were identified in ~12–13% of pediatric AML cases (n=13). Patients presented with aggressive disease biology and high early treatment burden. Most patients required multiple cycles of induction chemotherapy to achieve remission. Despite therapy, outcomes remained suboptimal: • Deaths: 6 patients (≈46%) • Relapses: 3 patients (≈23%) • Refractory disease: 7 patients (≈54%) Less than half of patients achieved sustained remission. Even among responders, durability of response was limited. Co-mutations (including NPM1, WT1, NRAS) were frequently observed, suggesting additional biological complexity contributing to treatment resistance. Notably, access to FLT3 inhibitors and timely hematopoietic stem cell transplantation was limited, potentially amplifying adverse outcomes. Overall survival remained markedly inferior compared to expected outcomes reported in high-income settings.

Conclusion: FLT3-mutated pediatric AML in our setting is associated with alarmingly high rates of treatment failure and death, underscoring a critical survival gap. These data provide compelling real-world evidence for: • urgent integration of FLT3-targeted therapies, • prioritization of early transplant strategies, and • development of resource-adapted treatment protocols. Bridging this therapeutic disparity is essential to improve survival in this biologically high-risk yet potentially targetable subgroup.

Abstract 116

Bone marrow adipose compartment in patients with hematologic malignancies

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Purpose: Bone marrow adipose tissue is no longer viewed merely as a "filler" of space, but as an active participant in the tumor microenvironment. Under the combined influence of the malignancy and anti-cancer agents, there is an increase in bone marrow adipose tissue volume. The aim of the study is to evaluate the bone marrow adipose fraction in patients in remission from hematologic malignancies.

Methods: Bone marrow fat fraction analysis was performed using a 1.5T GE scanner with the IDEAL sequence and a 3T Philips scanner with the mDIXON-Quant sequence. A total of 43 patients from the Dmitry Rogachev National Medical Research Center of Pediatric Hematology, Oncology and

Immunology were randomly enrolled in the study. The cohort aged 6–17 years included patients with ICD-10 diagnoses C40–C96. The duration of remission ranged from 6 to 48 months.

Results: In all patients, the bone marrow fat fraction exceeded 45% in the L4 and L5 vertebral bodies. Furthermore, the bone marrow fat fraction in the iliac wings was found to be above 55% in all subjects. In 40 of our patients, the bone marrow fat fraction reached a disproportionate 80% in the L4 and L5 vertebral bodies and 90% in the iliac wings. Analysis of potential risk factors for bone marrow adipogenesis suggests a multicausal origin.

Conclusion: Examination of the bone marrow fat fraction in our patients following the completion of chemotherapy revealed a clear trend toward a disproportionate increase in bone marrow fat content.

Abstract 117

Clinical Profile and Outcomes of Philadelphia Chromosome-Positive Acute Leukemia in a Tertiary Care Center in Central India

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Purpose: Philadelphia chromosome-positive (Ph+) acute leukaemia is a rare in children (3-5% of pediatric ALL and 0.5-3% of pediatric AML). These are high-risk subtypes associated with poor prognosis, particularly in resource-limited settings. This study aims to describe the clinical characteristics, treatment patterns, and outcomes of Ph+ leukemia at a tertiary care center in central India.

Methods: A retrospective study was conducted, analyzing patients (aged 0–18 years) diagnosed with Ph+ leukemias between January 2019 and December 2025. Ph+ ALL/AML was confirmed by BCR-ABL1 fusion via conventional cytogenetics and/or polymerase chain reaction (PCR) or fluorescent in situ hybridisation (FISH). Data on demographics, clinical features, treatment regimens (chemotherapy, tyrosine kinase inhibitors [TKIs]), morphological remission, minimal residual disease [MRD], molecular remission, relapse, and mortality) were collected. Chronic myeloid leukemia with blast crisis was excluded.

Results: Of 280 acute leukemia patients screened, 19 (6.7%) had Ph+ status (17 B-ALL; one T-ALL and one AML. Among the Ph+ B-ALL, the median age of diagnosis was 7 years (2–15 years), and 65% were male. Common presentations included fever (89%), with hepatomegaly (88%) and splenomegaly (76%), with a mean spleen size of (1.9 ± 2.5 cm) below the left costal margin, and 35% had hyperleukocytosis with a median total leucocyte count (TLC) of (76×109/L). Only two patients had major (p210) BCR-ABL transcripts. Two patients denied treatment and one abandoned treatment. During 2nd week of induction chemotherapy as per the ICiCLe protocol, most of them received imatinib. After induction, all patients achieved morphological remission and 13 were MRD negative. Remaining 2 were also MRD negative after consolidation. Four had early relapse and seven died due to various causes.

Conclusion: Ph+ acute leukemia remains an uncommon but high-risk subtype. Despite high rates of remission and MRD negativity with TKI-based chemotherapy, early relapse and mortality rates are high.

Abstract 118

Clinical and Laboratory Characteristics and the Prognostic Value of LDH in Childhood Acute Leukemia at the Pediatric Center, Hue Central Hospital

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Purpose: Acute leukemia is the most common malignancy in children and adolescents. This study aimed to describe the clinical and laboratory characteristics of childhood acute leukemia and to evaluate the association between lactate dehydrogenase (LDH) levels and selected clinical and laboratory features.

Methods: A cross-sectional descriptive study was conducted in children diagnosed with acute leukemia at the Pediatric Center, Hue Central Hospital, from January 1, 2022 to April 30, 2023.

Results: A total of 48 patients with acute leukemia were included, of whom 16 had acute myeloid leukemia and 32 had acute lymphoblastic leukemia. The mean age was 5.3 ± 4.3 years, and the male-to-female ratio was 1:1.1. The most common clinical manifestations were anemia (75.0%), fever (56.2%), and hepatosplenomegaly (54.2%). Hemoglobin <7.0 g/dL was found in 35.4% of patients, white blood cell count ≥50 × 10^9/L in 33.3%, absolute neutrophil count ≤500/μL in 60.4%, and platelet count <20 × 10^9/L in 31.2%. Elevated LDH was observed in 64.4% of patients, hyperuricemia in 31.2%, elevated liver enzymes in 41.7%, and increased C-reactive protein in 74.5%. Serum LDH levels were significantly associated with splenomegaly, white blood cell count, absolute neutrophil count, platelet count, uric acid levels, and liver enzyme levels ($p<0.05$). A statistically significant association was also found between LDH levels and risk stratification in acute lymphoblastic leukemia.

Conclusion: The most common clinical manifestations of childhood acute leukemia were anemia, fever, and hepatosplenomegaly. LDH may be a useful prognostic marker in childhood acute leukemia.

Abstract 119

THE FIRST EXPERIENCE OF STANDARDIZING CHEMOTHERAPY FOR LYMPHOMAS IN CHILDREN IN UZBEKISTAN

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Purpose: The standardization of lymphoma treatment is crucial for improving survival rates and the quality of life for patients. While in developed countries, childhood lymphoma survival rates exceed 95%, in Uzbekistan, until recently, this figure remained below 30% due to non-programmatic treatment. The primary challenges are the feasibility of administering high-dose chemotherapy, including high-dose methotrexate, and the capacity to manage infectious complications during periods of myelotoxic suppression.

Methods: Since June 2025, our Center has been implementing programmatic chemotherapy for various types of lymphomas in accordance with the clinical guidelines of the International Society of Paediatric Oncology (SIOP). The study included 23 patients: 5 girls and 18 boys. The median age was 8.9 years (range, 1.8 to 17 years). Seven patients had Hodgkin lymphoma (HL), six had Burkitt lymphoma, two had other high-grade B-cell lymphomas (HGBCL), four had ALK-positive anaplastic large cell lymphoma (ALCL), one had MALT lymphoma, and three had peripheral T-cell lymphomas (PTCL). The diagnosis was based on biopsy with histological, immunohistochemical, and molecular genetic testing, including fluorescence in situ hybridization (FISH) of the biopsy specimens. Positron emission tomography-computed tomography (PET-CT) was performed to assess disease stage initially and for follow-up.

Results: Of the patients with HL, three were diagnosed with stage 2, three with stage 3, and one with stage 4 disease. Among the patients with HGBCL, two were diagnosed with stage 2 and six with stage 3. Three patients with ALCL had stage 3 disease and one had stage 2. Two patients with PTCL had stage 3 and one had stage 2. The patient with MALT lymphoma had stage 3 disease. An initial response to therapy was achieved in all cases. Two patients (one with HGBCL and one with PTCL) died due to infectious complications; all other patients remain alive.

Conclusion: This represents the first experience with implementing standardized chemotherapy protocols for lymphomas in children in Uzbekistan. This therapy, including high-dose options, appears to be effective and feasible within the local context. Further follow-up studies are required to provide a more definitive evaluation.

Abstract 120

Role of Nutritional Supplementation Strategy in Prevention of Oral Mucositis while using High Dose Methotrexate in children with Acute Lymphoblastic Leukemia /Lymphoma: An interventional comparative study

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Purpose: Administration of HD-MTX (High Dose Methotrexate) in patients with Acute Lymphoblastic Leukemia/Lymphoma is associated with Oral Mucositis (OM). There is a paucity of specific research investigating the role of nutritional supplementation strategies in HD-MTX induced OM.

Methods: A total of 37 children (1–18 years) who completed all cycles of HD-MTX were enrolled. Prophylactic glutamine (400 mg/kg/day as swish-and-swallow) and elemental zinc (20 mg) were administered during HD-MTX in cycles 3 and 4 along with supportive care for 14 days. The incidence, severity, onset, and duration of OM, duration of febrile neutropenia, and hospital stay were compared between cycles 3–4 (with glutamine and zinc) and cycles 1–2 (supportive care alone) in the same patients.

Results: Children in cycle 3 and 4 showed a statistically significant (p= 0.028) reduction in incidence of OM (52.7%) as compared to those in the cycle 1 and 2 (70.3%). The maximum severity of pain reported was significantly lower in cycle 3 and 4 as compared to cycle 1 and 2. Similarly, the mean duration of oral mucositis was significantly reduced among participants in cycle 3 and 4 (M = 2.53, SD = 1.81) relative to those in the cycle 1 and 2 (M = 4.38, SD = 2.77), p = 0.007. The duration of febrile neutropenia and the mean length of hospital stay was significantly shorter in the cycle 3 and 4 compared to cycle 1 and 2 group. Furthermore, a significant reduction was observed in the duration of antibiotic use in the cycle 3 and 4 when compared with the cycle 1 and 2.

Conclusion: Glutamine and zinc in significantly reduces the effect of oral mucositis after cycle 3 and 4 of HD MTX in our study. However, further randomized controlled trials are needed with larger sample size for the definitive role of nutritional supplementation.

Abstract 121

TREATMENT OUTCOMES OF PEDIATRIC T-CELL LYMPHOMA AT HO CHI MINH CITY HEMATOLOGY AND BLOOD TRANSFUSION HOSPITAL

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Purpose: T-cell lymphoma is a group of malignant hematologic diseases that includes several subtypes in children, such as Anaplastic Large Cell Lymphoma (ALCL), T-cell Lymphoblastic Lymphoma (T-LL), Hepatosplenic T-cell Lymphoma (HSTCL), Peripheral T-cell Lymphoma (PTCL), and other rare variants. T-cell lymphomas are relatively rare, accounting for approximately 30% of non-Hodgkin lymphomas in children, and present major challenges in research, diagnosis, and treatment [1]. Furthermore, each subtype of pediatric T-cell lymphoma requires a specific approach for risk assessment and treatment. Pediatric T-cell lymphoma has been treated at Blood Transfusion and Hematology Hospital for several years, but until now, no study has evaluated treatment outcomes for this disease. Objective: To investigate the characteristics and summarize the treatment outcomes of pediatric T-cell lymphoma at Blood Transfusion and Hematology Hospital.

Methods: A retrospective, case series study. All patients under 16 years of age diagnosed with T-cell lymphoma at the Blood Transfusion and Hematology Hospital from 2017 to present were included. Data were analyzed by SPSS 20.

Results: During the study period, 13 patients were diagnosed and treated for T-cell lymphoma at Blood Transfusion and Hematology Hospital. ALCL was the most common subtype (38.5%), followed by T-LL (23.1%), PTCL-NOS (15.4%), Subcutaneous Panniculitis-like T-cell Lymphoma (SPTCL) (15.4%), and Systemic EBV-positive T-cell Lymphoma (S-EBV-TCL) (7.7%). All ALCL cases were ALK-positive and treated with the ALCL-99 protocol, with a complete response rate of 60%. All three T-LL patients responded to the FRALLE 2000T regimen, and no relapses were recorded during the study period. The two PTCL-NOS patients were initially treated with the CHOP/CHOEP protocol, with a 100% progression rate. The rarest case in the study, S-EBV-TCL case progressed and became resistant to treatments. The 2-year overall survival rate was 59.9±16.2%, and the 18-month event-free survival rate was 50.5±14.8%.

Conclusion: Among pediatric T-cell lymphomas, ALCL and T-LL are the most common, consistent with data from Vietnam and international publications. Except for T-LL, other subtypes such as ALCL, PTCL-NOS, SPTCL, and S-EBV-TCL have high rates of poor response (40–100%). The small number of patients is a limitation for evaluating treatment efficacy in very rare subtypes like SPTCL and S-EBV-TCL. The treatment approaches for pediatric T-cell lymphoma at the Blood Transfusion and Hematology Hospital are consistent with those applied and updated at many cancer centers worldwide. However, significant challenges remain in treating these diseases. Targeted therapies and stem cell transplantation are still being studied for their effectiveness.

Abstract 122

PERSISTENT MICROCYTIC ANEMIA AFTER REMISSION OF PEDIATRIC ACUTE LYMPHOBLASTIC LEUKEMIA REVEALING b-THALASSEMIA TRAIT: A CASE REPORT

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Purpose: The coexistence of leukemia and thalassemia patients has rarely been reported. The presence of underlying hemoglobinopathy may interfer with the interpretation of hematologic findings during the diagnosis and management of leukemia.

Methods: We present a pediatric case in which b-thalassemia trait was identified after remission, prompted by persistent microcytic anemia.

Results: We describe a 4-year-5-month-old girl diagnosed with acute lymphoblastic leukemia (ALL). Initial clinical features showed pancytopenia accompanied with pallor, joint pain, and petechiae in extremities. Peripheral blood smear demonstrated marked anisocytosis, with microcytic, hypochromic erythrocytes, and significant poikilocytosis including spherocytes, ovalocytes, target cells, teardrop cells, cigar cells, pencil cells, and fragmented erythrocytes. Bone marrow aspiration revealed hypercellular marrow with predominance of lymphoblasts 23% showing morphological features consistent with ALL-L2. Immunophenotyping confirmed the diagnosis of B-lineage ALL. The patient underwent chemotherapy according to the Indonesian ALL standard-risk protocol and completed two years of chemotherapy with complete remission achievement. Hematopoietic response during chemotherapy was adequate, with leukocyte counts between 2,100 and 11,600/µL, and platelet counts ranging from 167,000 to 506,000/µL. However during post-therapy follow-up, erythropoiesis appeared suboptimal, as hemoglobin levels persistently remained below the normal range (7.1 to 11.1 g/dL) and erythrocyte morphology continued to demonstrate microcytic hypochromic features. Iron studies showed normal features with an elevated ferritin level. Subsequent electrophoresis revealed findings consistent with b- thalassemia trait with HbA 80.1%, HbF 15.1%, HbA2 4.8% and no detectable HbC, HbD , HbH, or HbE. The patient was therefore scheduled for continued follow-up and monitoring for both conditions.

Conclusion: Hemoglobinopathies should be considered in pediatric patients with hematologic malignancies who demonstrate an inadequate erythropoietic response despite appropriate therapy. Additionaly, individuals with underlying hemoglobinopathies may have an increased susceptibility to develop hematologic malignancies, highlighting the importance of comprehensive hematologic evaluation in such cases.

Abstract 123

Advanced Hemophilic Arthropathy Due to Recurrent Hemarthrosis in Moderate Hemophilia A: A Long-Term Clinical Course

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Purpose: Hemophilia A is an X-linked inherited bleeding disorder caused by deficiency of coagulation factor VIII. Recurrent hemarthroses remain the most important cause of chronic musculoskeletal morbidity, leading to progressive hemophilic arthropathy and functional disability. Although prophylactic therapies have improved outcomes, repeated bleeding episodes may still result in target joints and irreversible joint destruction. The aim of this report is to present the long-term clinical course and complications of recurrent hemarthroses in a patient with moderate Hemophilia A.

Methods: A retrospective clinical review was conducted of a 31-year-old male diagnosed with Hemophilia A in early childhood and followed regularly at a hematology center. Clinical records, bleeding history, joint involvement, laboratory findings, and therapeutic interventions were analyzed. Factor VIII activity ranged between 3% and 7.5%.

Results: The patient experienced recurrent bleeding episodes including epistaxis, gingival bleeding, hematuria, gastrointestinal bleeding, and multiple soft-tissue hematomas. Repeated hemarthroses predominantly involved the knees, right elbow, ankle, and hip joints, resulting in progressive hemophilic arthropathy and partial joint contractures. The right knee developed into a target joint with severe deformity and marked limitation of motion. Over time the patient required multiple joint aspirations, four chemical synovectomies, and hospitalization for large muscle hematomas. Additional complications included persistent bleeding after dental implantation and recurrent macrohematuria. Treatment included factor VIII replacement therapy, antifibrinolytic agents, and later prophylaxis with emicizumab. Due to advanced joint destruction and chronic hemarthrosis, knee reconstructive surgery was performed in 2024.

Conclusion: This case highlights the cumulative impact of recurrent hemarthroses leading to severe hemophilic arthropathy even in moderate Hemophilia A. Early prophylaxis, prevention of target joints, and timely use of modern therapies are essential to reduce long-term disability.

Abstract 124

Febrile Neutropenia During Maintenance Therapy in Pediatric Acute Lymphoblastic Leukemia: Real-World Data from a Tertiary Center in a Resource-Limited Healthcare Setting

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Purpose: Febrile neutropenia (FN) remains a frequent complication in children with acute lymphoblastic leukemia (ALL); however, data focusing specifically on FN occurring during maintenance therapy remain limited, particularly in low- and middle-income settings. This study aimed to determine the incidence, clinical characteristics, treatment-related factors, and outcomes of FN in patients with ALL during the maintenance phase in a new Vietnamese Pediatric Oncology center.

Methods: We conducted a descriptive retrospective study of 97 children with ALL who completed maintenance therapy at a tertiary center in Ho Chi Minh City (2020–2025).

Results: Thirty patients (30.9%) experienced 44 episodes of febrile neutropenia. FN episodes were characterized by profound neutropenia, with 25% presenting with severe neutropenia (ANC ≤0.1 $\times 10^3/\mu L$). The median duration of hospitalization was 11 days (IQR 7–17), and 52.3% of episodes required hospital stays longer than 10 days. Higher 6-mercaptopurine dosing was significantly associated with the occurrence of FN ($p = 0.018$). Microbiologically documented infections were identified in 18.1% of episodes through positive blood cultures. ICU admission occurred in 9.1% of episodes, and in-hospital mortality was 13.6%. Intermediate metabolizer variants of NUDT15 were identified among tested patients, while TPMT variants were uncommon.

Conclusion: Febrile neutropenia during maintenance therapy remains a clinically relevant complication in children with ALL. The observed association between higher 6-mercaptopurine dosing and FN highlights the need for careful treatment monitoring during maintenance therapy

Abstract 125

Management Challenges In Secondary Antiphospholipid Syndrome Following Splenectomy

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Purpose: Secondary antiphospholipid syndrome (APS) causes recurrent thrombosis and often overlaps with hematologic disorders. Splenectomy heightens thrombotic/infectious risks. Optimal anticoagulation in high-risk APS with unstable INR is debated. We report post-splenectomy therapeutic challenges in a complex case.

Methods: A 72-year-old woman with hemolytic anemia and secondary APS was admitted 20 days post-splenectomy. History: recurrent thoracic arterial emboli (four episodes, 2021–2024) and splenic infarctions (2023, 2025). Warfarin stopped due to INR instability (peak 8.0) and hemorrhage. Current regimen: rivaroxaban 15 mg daily, aspirin 100 mg daily, hydroxychloroquine 400 mg daily, amoxicillin/clavulanic acid 875 mg BID. Labs assessed blood count and coagulation.

Results: Hemoglobin 104 g/L, RBC $3.54 \times 10^6/\mu L$, WBC $7.19 \times 10^3/\mu L$, platelets $718 \times 10^3/\mu L$ (anemia, reactive thrombocytosis). Coagulation: INR 0.95, aPTT 39.3 s, fibrinogen 5.27 g/L, D-dimer 2.24 µg/mL (hypercoagulability). Rivaroxaban continued with monitoring; potential VKA transition considered. Hydroxychloroquine/aspirin maintained. Post-splenectomy vaccinations initiated (pneumococcal, meningococcal, Hib, influenza). Follow-up: serial blood counts, coagulation, D-dimer, antiphospholipid antibodies.

Conclusion: Post-splenectomy secondary APS with recurrent arterial thrombosis is ultra-high-risk. INR instability precluded VKAs, requiring tailored DOAC strategy. Thrombocytosis and immunosuppression amplified risks. This case emphasizes personalized multidisciplinary care and unresolved DOAC role in high-risk APS.

Abstract 126

Mechanism of ICAM1-Mediated Leukemic Cell Proliferation in Acute Lymphoblastic Leukemia via the Notch1-P38 Signaling Pathway

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Purpose: This study aims to explore the prognostic value of Intercellular Adhesion Molecule-1 (ICAM-1/CD54) in Acute Lymphoblastic Leukemia (ALL). It further investigates the expression profile of ICAM-1 in ALL and elucidates the potential molecular mechanisms by which ICAM-1 influences the proliferation and migration of leukemic cells through the Notch1-P38 signaling pathway.

Methods: Clinical Sample Analysis: Bone marrow samples were collected from patients with B-cell Acute Lymphoblastic Leukemia (B-ALL) and patients with non-malignant hematological diseases. RNA sequencing (RNA-seq) and RT-qPCR were utilized to detect ICAM1 expression levels, and its correlation with clinical characteristics and prognosis (PFS, OS) was analyzed. Cell Culture and Modification: Human B-ALL cell lines Nalm6 and Ball-1 were selected. Stable cell lines with ICAM1 overexpression and knockdown were constructed via lentiviral transfection. Functional Assays: Cell proliferation was assessed using CCK-8 and EdU assays. Flow cytometry was used to detect cell apoptosis, and Transwell assays were performed to evaluate cell invasion and migration capabilities. Mechanism Investigation: Western Blotting was used to detect the expression of pathway-related proteins, and Co-immunoprecipitation (Co-IP) was employed to verify interactions between molecules. In Vivo Validation: A subcutaneous xenograft tumor model in nude mice was established to observe the effects of ICAM1 on tumor growth and survival rate in vivo.

Results: Clinical Expression: ICAM1 expression was significantly elevated in the bone marrow of pediatric patients with relapsed/refractory B-ALL. High ICAM1 expression correlated positively with high white blood cell counts at initial diagnosis and specific genetic mutations (e.g., KRAS, IKZF1). Prognostic Value: Patients in the high ICAM1 expression group showed significantly shorter progression-free survival (PFS) (P=0.01), identifying ICAM1 as an independent risk factor for poor prognosis. Cellular Function: In vitro experiments demonstrated that ICAM1 overexpression significantly promoted the proliferation and invasion of Nalm6 and Ball-1 cells, while ICAM1 knockdown inhibited proliferation and induced apoptosis. Signaling Pathway: Overexpression of ICAM1 activated the Notch1-P38 signaling pathway, leading to the upregulated expression of downstream oncogenic genes.

Conclusion: ICAM1 promotes leukemic cell proliferation and invasion while inhibiting apoptosis in B-ALL through the activation of the Notch1-P38 signaling pathway. High expression of ICAM1 is closely associated with malignant progression and poor prognosis in B-ALL, suggesting it may serve as a valuable biomarker for prognostic assessment and a potential therapeutic target in clinical treatment.

Abstract 127

When Immune Thrombocytopenia Is Not Immune: A Case Revealing MYH9-Related Disease

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Purpose: Inherited thrombocytopenias may be misdiagnosed as immune thrombocytopenia (ITP), particularly when thrombocytopenia presents in early childhood and persists despite therapy. We aim to present a case of long-standing thrombocytopenia initially treated as chronic ITP that was later identified as MYH9-related disease after genetic testing.

Methods: We describe the clinical course, laboratory findings, treatment history, and genetic investigation of a patient with long-standing thrombocytopenia initially diagnosed as chronic ITP.

Results: Thrombocytopenia was first detected at the age of three years and confirmed by bone marrow examination. The patient received multiple treatments including corticosteroids (dexamethasone and later prednisolone), thrombopoietin receptor agonist (eltrombopag), and intravenous immunoglobulin, with only partial and transient responses. Due to persistent thrombocytopenia and steroid resistance, splenectomy was performed in 2018, resulting in temporary improvement of platelet counts. The patient experienced recurrent bruising, petechiae, and occasional mucosal bleeding. During two pregnancies, thrombocytopenia worsened and corticosteroid therapy showed limited efficacy. After splenectomy, the patient received recommended vaccinations and antibacterial prophylaxis. A transient episode of pancreatitis occurred post-splenectomy but later resolved. Further evaluation demonstrated defective CD42 binding, while antiphospholipid syndrome, viral hepatitis, and hepatosplenomegaly were excluded. Given the lifelong thrombocytopenia, progressive hearing loss, and thrombocytopenia in the second child, inherited thrombocytopenia was suspected. Genetic testing in the child identified a MYH9 (Arg702Cys) mutation, confirming MYH9-related disease.

Conclusion: This case highlights the importance of considering inherited thrombocytopenia in patients with lifelong thrombocytopenia and poor response to immunosuppressive therapy. MYH9-related disease should be suspected when thrombocytopenia is associated with extra-hematologic manifestations such as hearing loss. Early genetic testing may prevent unnecessary treatments and invasive procedures.

Abstract 128

Atypical Budd–Chiari Syndrome In An Adolescent With Secondary Hypocoagulation And Portal Hypertension

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Purpose: Budd–Chiari syndrome (BCS) is a rare hepatic vascular disorder due to hepatic venous outflow obstruction, often linked to hypercoagulable states. Pediatric cases are rare, typically involving inherited thrombophilia or vascular anomalies. We report an atypical adolescent case with secondary hypocoagulation, portal hypertension, and no initial thrombosis.

Methods: A 16-year-old male presented with exertional subcostal pain and abdominal discomfort. Exam showed hepatomegaly (+2 cm) and massive splenomegaly (+14 cm). Labs revealed thrombocytopenia (65.8 × 10^9/L) and leukopenia (3.15 × 10^9/L), worsening to 46.0 × 10^9/L and 2.88 × 10^9/L after four months. Coagulation was abnormal: PT 17–18.8 s, aPTT 38.3–40.2 s, INR 1.56–1.77, fibrinogen 2.22 g/L, factor VII 33%, factor V 29%. Imaging confirmed splenomegaly (24.8 × 8.0 cm), enlarged portal vein (2.2 cm), dilated mesenteric/splenic veins (up to 2.1 cm), and portal hypertension without thrombosis or collaterals. Liver fibrosis was METAVIR F3.

Results: Bone marrow trepanobiopsy showed reactive changes, no myelofibrosis. Splenectomy (pre-op platelets 50 × 10^9/L) yielded postoperative thrombocytosis (907 × 10^9/L) and elevated D-dimer (5.99 µg/mL), with 30% portal confluence thrombosis. Spleen histopathology revealed reactive white pulp depletion and active germinal centers (high Ki-67).

Conclusion: This adolescent BCS case featured secondary hypocoagulation and infectious mononucleosis-related splenomegaly, not hypercoagulability. Absent initial thrombosis and inherited fibrosis complicated diagnosis/management. Multidisciplinary care and surveillance are crucial for non-classical pediatric BCS, stressing tailored hemostatic evaluation in thrombo-immune liver disorders.

Abstract 129

Nodular Lymphocyte Predominant Hodgkin Lymphoma (NLPHL) in Children: Clinico-pathological Profile, Treatment Outcomes and Impact of Histological Pattern — A Single-Centre Retrospective Analysis

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Purpose: NLPHL is a rare indolent B-cell lymphoma (<5% of paediatric Hodgkin lymphoma) prone to late relapse and histological transformation, with limited paediatric data.

Methods: We retrospectively analysed 32 paediatric NLPHL patients at a single tertiary care cancer centre (January 2021–March 2026). Treatment was risk-adapted: Stage I non-bulky completely excised disease was observed (n=3); limited-stage(I–II) symptomatic/bulky disease received chemotherapy (n=14); advanced-stage(III–IV) disease received rituximab-based chemo-immunotherapy (n=15); no patients received radiotherapy.

Results: All 32 patients (male:female15:1) with a median age of 11.5(3.5–14) years underwent excision biopsy for diagnosis, 22(69%) and 10(31%) had limited (I-13;II-9) and advanced (III-3,IV-7) stage disease respectively. B-symptoms, bulky disease, and extranodal involvement were seen in 7 (22%), 9 (28%), and 9(28%) respectively; extranodal sites were bone marrow (n=6), spleen (n=4), skeletal, muscle/soft tissue, peritoneum (n=1 each). Histology was Typical (A/B) in 15 (47%) and Variant (C–F) in 17 (53%). Variant pattern had significantly greater extranodal involvement (47% vs 7%; OR 12.44, 95% CI 1.32–117.04; p=0.018) and a trend toward advanced-stage disease (47% vs 13%; OR 5.78; p=0.061). Treatment regimens included chemotherapy-only approach- CVP (n=11) and chemo-immunotherapy: R-CHOP (n=7), R-MINE (n=4), others (n=4). Treatment-related toxicities occurred in 6 (18.8%) with no treatment-related mortality. Two (EBV-positive and negative 1 each) transformed to DLBCL, both being stage IV Variant E-pattern; the EBV-negative patient was salvaged with rituximab-based chemo-immunotherapy and the EBV-positive patient with nivolumab-based salvage followed by autologous transplant, both are in remission at last follow-up, confirming curability after histological progression. Overall 2-year EFS was 89.1% (95% CI 73.9–100%); limited-stage 100% vs advanced-stage 67.5% (p=0.063). At median follow-up of 21.5 months (range 2–60), all 32 are alive (OS 100%).

Conclusion: Paediatric NLPHL is a rare, heterogeneous disease requiring tailored treatment.Risk-adapted therapy achieves excellent outcomes with minimal

toxicity.

Abstract 130

Dynamic DNA methylation changes during disease course in juvenile myelomonocytic leukemia

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Purpose: Juvenile myelomonocytic leukemia (JMML) is a rare myelodysplastic/myeloproliferative neoplasm that arises during infancy and early childhood. Aberrant DNA methylation has been identified as an independent prognostic factor in JMML. However, studies investigating the dynamic changes of DNA methylation throughout disease progression, including remission and relapse, remain limited.

Methods: DNA methylation was assessed in 15 JMML patients. Two patients were profiled using the Illumina Infinium MethylationEPIC 850K BeadChip (EPIC array), while the remaining 11 patients were analyzed by targeted bisulfite sequencing of 59,855 JMML-related CpG sites using next-generation sequencing (NGS). Site-level gene set enrichment analysis (GSEA) was performed with Hallmark pathways, using CpG methylation changes as the ranking metric to evaluate pathway-level DNA methylation alterations across treatment stages. For the 13 patients with paired bone marrow (BM) and peripheral blood (PB) samples, Pearson correlation coefficients were calculated to assess concordance between BM and PB methylation profiles.

Results: Pearson correlation coefficients between bone marrow (BM) and peripheral blood (PB) methylation profiles were >0.85 in all patients, with nine exceeding 0.90, indicating high concordance and supporting the use of PB as a surrogate for longitudinal methylation analysis. In three patients monitored from diagnosis to remission, genome-wide CpG methylation levels decreased significantly (median $\Delta\beta$ = -0.098, mean $\Delta\beta$ = -0.102, Wilcoxon $P < 2\times10^{-16}$), indicating a global hypomethylation trend during remission. Conversely, in three patients followed from remission to relapse, methylation levels increased significantly (median $\Delta\beta$ = 0.086, mean $\Delta\beta$ = 0.102, Wilcoxon $P < 2\times10^{-16}$), suggesting global remethylation at relapse. Longitudinal 850K methylation analysis in two patients across diagnosis, remission, and relapse revealed that certain promoter CpG sites underwent demethylation during remission and re-methylation at relapse. The number of reversible promoter CpG sites was 13,260 for P12 (18.2% of total promoter CpGs) and 4,884 for P13 (6.7%), with 3,771 overlapping sites. Multi-timepoint NGS analysis of three patients showed stable genome-wide methylation during remission in two patients with sustained remission, whereas the patient who relapsed exhibited pronounced methylation fluctuations at relapse, followed by re-stabilization upon re-remission. In the patient exhibiting methylation fluctuations at relapse, clustering of highly variable CpG sites identified two epigenetic subgroups (Cluster 2 and Cluster 6) with reversible, disease state–dependent methylation changes, characterized by relative hypermethylation at diagnosis and relapse and demethylation during remission. GSEA revealed enrichment of EMT, developmental, and KRAS-related pathways in Cluster 2, while Cluster 6 was enriched for EMT-associated gene sets.

Conclusion: Peripheral blood methylation closely mirrors bone marrow, supporting its use for longitudinal monitoring. In one patient with relapse-associated methylation fluctuations, reversible CpG sites in Clusters 2 and 6 may be linked to relapse and could serve as potential biomarkers for disease progression.

Abstract 131

DEMOGRAPHIC PROFILE AND INCIDENCE OF HYPERTENSION IN PEDIATRIC PATIENTS WITH ACUTE LYMPHOBLASTIC LEUKEMIA DURING INDUCTION CHEMOTHERAPY: EXPERIENCE FROM A TERTIARY CANCER CARE CENTRE

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Purpose: Acute lymphoblastic leukemia (ALL) is the most common childhood malignancy with survival rates approaching 90%. Hypertension during induction chemotherapy is a recognized complication leading to serious sequelae like renal dysfunction and posterior reversible encephalopathy syndrome (PRES) if not treated promptly. However, data from North-East India are limited. This study aims to determine the incidence of hypertension during induction chemotherapy in pediatric patients with ALL and to identify demographic and clinical factors associated with its development.

Methods: This retrospective observational study was conducted in the Department of Pediatric Oncology at Dr. B. Borooah Cancer Institute, Guwahati, India from July 2022 to July 2024. A total of 195 patients aged 1-19 years with newly diagnosed ALL receiving induction chemotherapy were included. Patients with pre-existing hypertension or relapsed ALL were excluded. Blood pressure was recorded at baseline and during induction on days 1, 8, 15, 22 and 30. Hypertension was defined according to the 2017 American Academy of Pediatric guidelines. Logistic regression analysis was performed to identify predictors of hypertension.

Results: Hypertension developed in 32.8% patients during induction chemotherapy, majority(89.1%) developing within first 15 days of induction. Peak mean systolic and diastolic blood pressures observed on day 15 (124.9 ±11.3 mmHg and 85.14 ± 9.75 mmHg). Multivariate logistic regression identified tumour lysis syndrome (OR 2.41, p=0.015) and bulky kidneys on ultrasonography (OR 2.67, p=0.008) as independent predictors of hypertension. PRES occurred in 2 patients (3.1%). Majority (71.9%) patients were managed successfully with single-agent amlodipine, and no mortality related to hypertensive complications was observed.

Conclusion: Hypertension is a common early complication during induction chemotherapy in pediatric ALL patients. Routine blood pressure monitoring and early intervention are crucial to prevent complications and optimize supportive care.

Abstract 132

Acute T-lymphoblastic leukemia in children: the experience of the treatment protocol ALL-MB-2015 in Uzbekistan

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Purpose: T-cell acute lymphoblastic leukemia (T-ALL) accounts for approximately 10% of all cases of acute lymphoblastic leukemia (ALL) in children and has distinct biological characteristics. The aim of this study was to evaluate the effectiveness of therapy and the impact of various prognostic factors in patients with T-ALL treated according to the ALL-MB-2015 protocol.

Methods: The results of therapy in 130 patients with T-ALL treated according to the ALL-MB-2015 protocol at the clinic of the Scientific and Practical Medical Center of Oncology, Hematology and Immunology for the period 2020–2025 were analyzed. At diagnosis, 41 patients (32.2%) had a peripheral blood leukocyte count of less than 30×10^9/L, whereas in 50 patients (38.7%) it exceeded 100×10^9/L. In 58.1% of patients (76), the spleen extended less than 4 cm below the costal margin, while in 41.9% (54) it extended more than 4 cm. Co-expression of CD 13 and CD 33 was detected in 3 patients, and the t (6;11) MLL/AF6 translocation was identified in one patient.

Results: Among the 130 patients, death during induction due to infections occurred in 8 patients (6.4%), and one patient died due to disease progression. The induction mortality rate was 6.9% (9 patients). Three patients (2.3%) were non-responders, while complete remission was achieved in 118 patients (90.7%). Four patients (3.2%) were lost to follow-up (LFU), and relapse occurred in 25 patients (19.2%). The 3-year event-free survival (EFS) rate was 68.4% (89 patients). Event-free survival in patients with spleen size greater than 4 cm below the costal margin and an initial leukocyte count greater than 100×10^9/L was 53.8%, compared with 77.7% in patients with spleen size less than 4 cm and initial leukocytosis below 100×10^9/L. CD1a+ expression was detected in 88 patients (67.7%), and the EFS in this group was 71.4%. Co-expression of the myeloid antigens CD13 and CD33 was detected in 3 patients, and relapse occurred in all of them. In one patient, the t (6;11) MLL/AF6 translocation was identified, with a poor response on day 15 of induction (83% blasts); one year later this patient developed bone marrow relapse.

Conclusion: In this study, the factors associated with poor prognosis in T-ALL were leukocytosis above 100×10^9/L, spleen enlargement greater than 4 cm below the costal margin, poor response on day 15 of induction therapy, co-expression of myeloid antigens, and the presence of the t (6;11) MLL/AF6 translocation. CD1a expression was associated with a favorable prognosis.

Abstract 133

Primary Mediastinal Large B-Cell Lymphoma in Children: Sequential Immunochemotherapy with Nivo + BV+ BEGEV for Relapsed/Refractory Disease

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Purpose: Primary mediastinal Large B-cell lymphoma (PMBCL) is a rare (1–2% in children) aggressive NHL subtype, with relapsed/refractory (r/r) disease in 10–20%. Recognized as a distinct entity in the WHO classification, PMBCL has unique features: NF-κB/JAK-STAT dysregulation and PD-L1 overexpression enabling immune evasion. First-line treatment failure (R-DA-EPOCH or R-B-NHL-BFM95) requires immunotherapeutic salvage regimens. Optimal second-line approaches and auto-HSCT are discuss. Aims To evaluate the efficacy and safety of second-line therapy for r/r PMBCL in children using nivolumab (Nivo) + brentuximab vedotin (BV) + bendamustine, gemcitabine, vinorelbine (BEGEV), based on two clinical cases.

Methods: Diagnosis of relapsed PMBCL in two clinical cases was confirmed by histopathology/ immunohistochemistry and ^{18}F-FDG PET/CT (Deauville criteria).

Results: Case 1 (boy, 16 y/o, PMBCL, stage III). Progression after 4 × R-DA-EPOCH. Salvage 2nd line therapy included 2× HD-MTX (5 g/m²) + R-ICE + nivolumab achieved partial response by PET/CT with following progression on therapy. 3rd line treatment regimen was 4 × Nivo + BV + BEGEV with complete response, which was consolidated by auto-HSCT. Patient is alive without disease signs more than 2 years. Case 2 (girl, 13 y/o, PMBCL, stage III). During first line treatment R-B-NHL-BFM95 (2 blocks) an acute kidney injury had been developed and following regimen switched to 4 × R-DA-EPOCH. A partial remission was achieved by PET/CT, but a relapse at 6 months after end of treatment had been developed. Following treatment included 4 × Nivo + BV+ BEGEV with complete response, consolidated by auto-HSCT. Patient is alive 1 month after auto-HSCT. In both cases during Nivo + BV+ BEGEV regimen there were no life-threatening treatment-related complications

Conclusion: A sequential regimen Nivo + BV+ BEGEV is an effective and safety, reaches a complete response in patients with r/r PMBCL and auto-HSCT consolidates anti-tumor effect.

Abstract 134

Prevalence and Risk Factors of Invasive Fungal Disease in Children with Hematological Malignancies: A Prospective Study in a Tertiary Care Hospital of Bangladesh

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Purpose: Invasive fungal disease (IFD) is a major cause of morbidity in children receiving chemotherapy for hematological malignancies. The burden is particularly high in low- and middle-income countries where diagnostic resources and antifungal prophylaxis are limited. Prospective data from South Asia remain scarce. This study aimed to determine the prevalence, pathogen distribution, and risk factors for IFD in children with hematological malignancies in Bangladesh.

Methods: A prospective observational study was conducted between May 2024 and April 2025 at the Department of Pediatric Hematology and Oncology, Bangladesh Medical University. Seventy-six children younger than 18 years with hematological malignancies and suspected infection were consecutively enrolled. IFD was classified as proven, probable, or possible according to the EORTC/MSGERC 2020 criteria. Clinical, laboratory, microbiological, and radiological data were collected. Associations between potential risk factors and IFD were analysed using chi-square tests, analysis of variance, and multivariate logistic regression.

Results: The prevalence of proven or probable IFD was 17.1% (10.5% proven and 6.6% probable). An additional 19.7% of patients met criteria for possible IFD, resulting in an overall IFD burden of 36.8%. Candida species accounted for 61.5% of confirmed fungal isolates, while Aspergillus species represented 15.4%. On multivariate analysis, prolonged neutropenia longer than seven days was the strongest independent predictor of IFD (odds ratio 25.8, p=0.003). Elevated C-reactive protein was also independently associated with IFD (odds ratio 1.009 per unit increase, p=0.013). Chest pain (odds ratio 9.15, p=0.023) and mucositis (odds ratio 2.47, p=0.016) were significant clinical predictors.

Conclusion: IFD represents a substantial infectious burden among children with hematological malignancies in this resource-limited setting. Prolonged neutropenia, mucositis, chest pain, and elevated inflammatory markers were key predictors. Strengthening early diagnosis and implementing risk-based antifungal strategies may reduce infection-related morbidity in pediatric oncology programs in low-resource countries.

Abstract 135

Universal Health Coverage and Pediatric Leukemia Care: A Comparison of before and after Universal Health Coverage Implementation in Indonesia

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Purpose: Indonesia implemented the National Health Insurance program (Jaminan Kesehatan Nasional, JKN) in 2014 to improve access to healthcare. Its impact on pediatric oncology care remains incompletely understood. This study compares demographic, socioeconomic, and clinical characteristics of children with acute lymphoblastic leukemia (ALL) before and after JKN implementation.

Methods: A retrospective comparative analysis was conducted on children diagnosed with ALL in a tertiary referral hospital. Patients diagnosed in the pre-JKN period (2010–2013) were compared with those diagnosed in the post-JKN period (2019–2021). Variables analyzed included demographics, parental socioeconomic indicators, residence distance, insurance coverage, treatment protocols, comorbidities, and clinical outcomes.

Results: A total of 580 children with ALL were included (pre-JKN n=294; post-JKN n=286). Sex and age distributions were similar between periods, with most patients aged 1–<10 years. Insurance coverage changed substantially following JKN implementation, with uninsured patients decreasing dramatically while most patients became covered by contributory or subsidized national insurance. The proportion of patients living more than 100 km from the treatment center increased in the post-JKN period, suggesting improved geographic access to tertiary care. Documentation of parental education and occupation improved markedly after JKN implementation, with fewer missing socioeconomic data. Treatment protocols also evolved over time, shifting from the Indonesian ALL 2006 protocol to the Acute Lymphoblastic Leukemia 2016 protocol. Higher rates of reported comorbidities, mortality, and relapse were observed in the post-JKN period; however, these findings coincided with a substantial reduction in unknown outcome data, suggesting improved follow-up and reporting.

Conclusion: Implementation of Indonesia's national health insurance program was associated with expanded insurance coverage, improved access to tertiary pediatric oncology services, and better clinical documentation. While mortality and relapse appeared higher in the post-JKN period, this likely reflects improved data completeness rather than worsening outcomes. Continued evaluation is needed to determine the long-term impact of universal health coverage on survival among children with ALL.

Abstract 136

Prevalence and Risk Factors of Invasive Fungal Disease in Children with Hematological Malignancies: A Prospective Study from Bangladesh

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Purpose: Invasive fungal disease remains a major cause of morbidity in children receiving chemotherapy for hematological malignancies, particularly in low and

middle income countries where diagnostic and preventive resources are limited. Prospective data from South Asia are scarce. This study aimed to determine the prevalence, pathogen distribution, and risk factors of invasive fungal disease in pediatric hematology patients in Bangladesh using standardized diagnostic criteria.

Methods: A single centre prospective observational study was conducted from May 2024 to April 2025 at the Department of Pediatric Hematology and Oncology, Bangladesh Medical University. Seventy six children younger than 18 years with hematological malignancies and suspected infection were enrolled consecutively. Invasive fungal disease was classified as proven, probable, or possible according to EORTC Mycoses Study Group Education and Research Consortium 2020 criteria. Clinical, laboratory, microbiological, and radiological data were collected. Associations were analysed using chi square test, analysis of variance, and logistic regression.

Results: The overall prevalence of invasive fungal disease was 36.8 percent. Possible infection occurred in 19.7 percent, probable in 6.6 percent, and proven in 10.5 percent of patients. Candida species accounted for 61.5 percent of confirmed fungal isolates, while Aspergillus species were identified in 15.4 percent. Acute myeloid leukemia was significantly associated with invasive fungal disease. Severe prolonged neutropenia, elevated C reactive protein, mucositis, and chest pain were significant risk factors. Radiological abnormalities were more frequent in proven or probable cases. Duration of fever was significantly longer in children with confirmed infection.

Conclusion: More than one third of children with hematological malignancies developed invasive fungal disease in this resource limited setting. Prolonged neutropenia and inflammatory markers were strong predictors. Strengthening early diagnosis and risk based antifungal strategies is essential to reduce infectious morbidity in pediatric oncology patients in low resource countries.

Abstract 137

Persistent Voice Hoaerseness as a Clinical Manifestation of Soft Tissue Infiltration in Pediatric T-Cell Acute Lymphoblastic Leukemia Late Relaps

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Purpose: Background : T–cell acute lymphoblastic leukemia represents (ALL) approximately 12–15% of newly diagnosed pediatric ALL cases. Approximately 10–30% relapse cases occurred within 1–2 years of diagnosis. The most common extra–medullary isolated relapse is central nervous system while supraglottic mass is a rare soft tissue infiltration.

Methods: A case report of 15-year-old girl with persistent voice hoarseness for 4 months. Her voice hoarseness did not resolve with antibiotic or anti–inflammatory medication. There was no history of bone or joint pain. There was no liver or spleen enlargement during physical examination. She was diagnosed as pediatric ALL treated with 2016 Indonesia standard risk protocol. She achieved remission in 2022 with unremarkable routine complete blood count monitoring.

Results: Cervical CT–Scan showed supraglottic mass around 2.5 cm with malignant features and airway patency of 73%. Tissue biopsy and immunohistochemical staining confirmed T–cell ALL infiltration. There was no infiltration on surrounding tissue. Lymphoblast and cytopenia were not found on her peripheral blood examination. Bone marrow puncture and immunophenotyping were planned to confirm any extramedullary relapse.

Conclusion: Extra–medullary soft tissue relapse of T–cell ALL is a rare case. Chemotherapy was treatment of choice to eliminate leukemic cells in soft tissue infiltration. Serial imaging was planned to monitor its size. Airway patency measurement and irradiation should be prepared within the treatment plan. The outcome of treatment and evaluation will be presented during oral presentation.

Abstract 138

Impact of Clinical Setting in the Outcome of Pediatric Acute Myeloid Leukemia from year 2010 2023

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Purpose: In the Philippines, survival rate of patients with acute myeloid leukemia (AML) is yet to be documented, especially in Mindanao. This study aims to evaluate the impact of establishing a dedicated pediatric cancer facility, the Children's Cancer Institute (CCI), on patient outcomes in a government tertiary center.

Methods: A retrospective cohort study compared outcomes of AML patients treated with the Modified MRC 10 protocol across two periods: the CCBDU era (2010–2016) and the CCI era (2017–2023). The primary endpoints were overall survival (OS) and event-free survival (EFS) at one-year post-chemotherapy.

Results: Ninety-five patients were analyzed (CCBDU n=33; CCI n=62), with mean age of 9.7 years and male predominance (57.9 %). The age, sex and proportion of completed treatment did not differ significantly between the two groups. While remission rates post-induction (CCBDU 84% vs CCI 73.8%, p=0.19) and median time to relapse (242 vs 248 days) were comparable, the causes of mortality shifted significantly. Progressive disease was the primary cause of death in CCBDU (35.3%), whereas septic complications became the leading cause in CCI (33.6%, p=0.04). Notably, 27.4% of patients in the CCI era died while in remission (p

Conclusion: The establishment of a dedicated cancer facility did not automatically translate to a survival advantage, as septic complications emerged as the primary cause of mortality in the later cohort from CCI. Strengthening supportive care and infection management is recommended to improve AML survival in resource-limited settings.

Abstract 139

Pediatric Oncology Center of Azerbaijan's achievements in treating children with Non-Hodgkin lymphoma

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Purpose: In this article we analyzed the results of treatment of 116 children with NHL, who received treatment at the Pediatric Oncology Center of Azerbaijan.

Methods: Among the patients with the non-hodgkin lymphoma, Burkitt's lymphoma accounted for 62 pts, lymphoblastic lymphoma - 25, diffuse large B-cell lymphoma - 21, others - 8. Treatment was carried out according to protocols B-NHL-BFM -2004, ALL IC - BFM 2002 and ALCL 99. The patients with the mature B-cell NHL, in stage 3 and 4, received methotrexate 1gr + rituximab 375mg/m2.

Results: There were no toxic deaths. OS and EFS for all patients were 80% ±4% and 78% ±4%, respectively.

Conclusion: The implementation of modern and modified international protocols has led to a significant improvement in long-term treatment results of children with NHL in Azerbaijan.

Abstract 140

FACTORS PREDICTING EMPIRICAL ANTIBIOTIC ESCALATION IN PATIENTS WITH HEMATOLOGICAL MALIGNANCIES EXPERIENCING THEIR INITIAL EPISODE OF FEBRILE NEUTROPENIA AT DR. SARDJITO HOSPITAL, YOGYAKARTA, INDONESIA

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Purpose: Neutropenic fever is an oncology emergency. The incidence of neutropenic fever in hematological malignancy patients is 80%, with a mortality rate of up to 10%. Broad-spectrum empirical antibiotics must be promptly administered to reduce mortality rate and complications. The selection of empirical antibiotic types should consider the possible causative pathogens based on local microbial patterns. Antibiotic escalations are one of the indicators of empirical antibiotic failure, which is quite common in patients with febrile neutropenia in hematological malignancies. Aim: To determine predictor factors of empirical antibiotic escalation in patients with hematological malignancies with the first episode of febrile neutropenia.

Methods: A retrospective cohort study was conducted in patients with febrile neutropenia in hematological malignancies at Sardjito Hospital Yogyakarta

between January 2018 and December 2022, samples were selected by consecutive sampling. Bivariate analysis was performed using Chi-square test, independent variables with a p-value < 0.25 continued to multivariate analysis using the logistic regression test with a significance value of p < 0.05. The strength of associations was determined using adjusted odds ratios (aOR) and 95% confidence intervals (CIs).

Results: A total of 397 patients with first-episode febrile neutropenia in hematological malignancies were treated during the study period. Among them, 223 subjects met the inclusion criteria, and 61 (27.4%) subjects experienced empirical antibiotic escalation. Multivariate analysis showed that pneumonia as a source of focal infection (p 0.003; aOR 3.09; 95% CI 1.46-6.56) was a significant predictor factor of empirical antibiotic escalation in hematology.

Conclusion: Pneumonia is a predictor factor of empirical antibiotic escalation in hematological malignancy patients with the first episode of febrile neutropenia.

Abstract 141

L-Asparaginase vs. Peg-Asparaginase in Pediatric Acute Lymphoblastic Leukemia: Induction Remission and Hypersensitivity in the Indonesian ALL 2024 Protocol

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Purpose: Asparaginase is a key component of induction therapy for pediatric acute lymphoblastic leukemia (ALL). Native L-asparaginase (L-ASP) and pegylated asparaginase (PEG-ASP) are commonly used, with PEG-ASP generally preferred due to its longer half-life and lower immunogenicity. PEG-ASP was introduced in our centre in June 2025. This study compared induction remission rates between L-ASP and PEG-ASP in children with ALL treated under the Indonesian ALL 2024 protocol and evaluated hypersensitivity reactions.

Methods: A retrospective cohort study was conducted among pediatric ALL patients treated at Dr. Sardjito Hospital between April 2024 and February 2026 using the Indonesian ALL 2024 protocol. Patients received induction therapy with either L-ASP or PEG-ASP. Induction remission rates were compared using the chi-square test, and risk ratios (RR) with 95% confidence intervals (CI) were calculated. Hypersensitivity reactions were also assessed.

Results: Sixty-seven patients were included, with 34 receiving L-ASP and 33 receiving PEG-ASP. Induction remission was achieved in 70.6% of the L-ASP group and 81.8% of the PEG-ASP group, with no statistically significant difference (p = 0.281; RR 0.86; 95% CI 0.66–1.13). Hypersensitivity reactions occurred in 6 of 33 patients (18%) receiving PEG-ASP. Data on hypersensitivity in the L-ASP group were insufficient for direct comparison. Most allergic reactions occurred during the early phase of PEG-ASP implementation, whereas sustained steroid dose and antihistamine premedication appeared to reduce hypersensitivity in subsequent cases.

Conclusion: PEG-ASP demonstrated a slightly higher induction remission rate than L-ASP, although the difference was not statistically significant. Premedication may reduce hypersensitivity and improve tolerability. With less frequent dosing and a manageable safety profile, PEG-ASP remains a promising option for induction therapy in pediatric ALL and may improve treatment adherence. However, the sample size did not meet the calculated minimum requirement, which may have limited statistical power.

Abstract 142

EARLY DEATH AMONG CHILDREN WITH NEUTROPENIC FEVER AND PSEUDOMONAS AERUGINOSA BACTEREMIA: EMPIRICAL ANTIBIOTIC SUSCEPTIBILITY MATTERS

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Purpose: Infection is the leading cause of death among children with cancers. Various clinical practice guidelines recommend the immediate administration of antibiotics with the Pseudomonas aeruginosa coverage in children with neutropenic fever. Our previous study showed multidrug resistance in 33% of Pseudomonas aeruginosa isolates obtained from children with neutropenic fever. It is, therefore, reasonable to disclose the death rate of Pseudomonas aeruginosa bacteremia for the development of local neutropenic fever guidelines and to associate it with the empirical antibiotic susceptibility.

Methods: Patients younger than 19 years of age with neutropenic fever, in accordance with the diagnostic criteria established by the Infectious Diseases Society of America, and Pseudomonas aeruginosa bacteremia admitted to Dr. Sardjito General Hospital during the period of 2022 – 2023 were eligible for this descriptive study. These patients were observed for the occurrence of death within 30 days of bacteremia. Day 0 of bacteremia was defined as the day of blood specimen collection for antibiotic susceptibility test.

Results: Eight cases of Pseudomonas aeruginosa bacteremia from seven patients (acute lymphoblastic leukemia, n = 3; acute myeloblastic leukemia, n = 3; germ cell tumor, n = 1) were identified during this study period. These cases received intravenous ciprofloxacin (n = 6) and ceftriaxone (n = 2) as the empirical antibiotics. Death was observed in three (38%) of eight cases (days 0, 1, and 5 of bacteremia, respectively). Ciprofloxacin-resistant Pseudomonas aeruginosa was found in one of the three deceased cases, whereas ceftriaxone, which reportedly have a limited coverage to Pseudomonas aeruginosa, was administered to two deceased cases. Among five survived cases, three cases showed the ciprofloxacin-sensitive Pseudomonas aeruginosa on antibiotic susceptibility tests.

Conclusion: Despite of its low incidence among children with cancers, Pseudomonas aeruginosa bacteremia was associated with an early death, particularly when the empirical antibiotics were not susceptible to this microorganism.

Abstract 143

Lineage Switch from B-Myeloid Ph+ to T-Myeloid Ph+ Acute Leukemia at Relapse: A Rare Case with Favourable Outcome

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Purpose: Mixed Phenotype Acute Leukemia (MPAL) with BCR-ABL1 gene rearrangements, is a rare and aggressive haematological malignancy in childhood, conferring a high-risk group and necessitating intensive chemotherapy. We report an exceptionally rare case of a paediatric patient initially diagnosed with MPAL (B+Myeloid, Ph+) who experienced a lineage switch to MPAL (T+Myeloid, Ph+) at first bone marrow relapse.

Methods: Diagnosis were performed according to current WHO classification criteria, incorporating morphological assessment, immunophenotyping, karyotype, FISH, PCR and cytochemical reactions.

Results: A 16-year-old male presented in April 2019 with anaemia (haemoglobin 52 g/dL), leukocytosis (25,000/µL), and thrombocytopenia (12,000/µL). Bone marrow aspirate demonstrated: myeloblasts (45%), promyeloblasts (8%), and L2-type lymphoblasts (25%). Immunophenotyping showed positivity for CD22, CD19, CD10, CD13, CD33, and HLA-DR; myeloperoxidase cytochemistry was positive. Cytogenetic analysis identified t(9;22)(q34;q11), and molecular analysis confirmed BCR-ABL1 P190 transcript. The case was classified as MPAL (B/Myeloid)+Ph. The patient was treated according to the ALL-MB-2015 protocol (Group E, BCR-ABL1 P190-positive ALL) with imatinib, followed by 2-year maintenance therapy. In December 2023, first bone marrow relapse was confirmed. Re-evaluation by FISH again demonstrated t(9;22)(q34;q11) BCR-ABL1, flow cytometry identified two distinct blast populations: acute myeloid leukaemia with CD7 co-expression, and 4% T-lineage was identified with the immunophenotype cytCD3+, CD7+, CD5+, CD4+, CD1a+. Cytochemical analysis showed myeloperoxidase positivity in 36% of blasts. The final diagnosis at relapse was: MPAL (T+Myeloid), Ph-positive, t(9;22)(q34;q11) BCR-ABL1, 1st bone marrow relapse (ICD-10: C94.0). Salvage therapy was initiated per the BFM ALL-REZ 2019 protocol with imatinib. Following the first intensive chemotherapy block, the patient achieved MRD-negative complete remission. He is currently continuing low-dose maintenance chemotherapy with imatinib in the outpatient setting.

Conclusion: The transformation from MPAL B/Myeloid Ph+ to MPAL T/Myeloid Ph+ at relapse represents an exceptionally rare phenomenon and underscoring the potential for cure in selected Ph+ MPAL patients through

chemotherapy and targeted agents alone.

Abstract 144

Prevalence of treatment-related mortality in children with Acute Lymphoblastic Leukemia in a tertiary hospital: a retrospective cross sectional study

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Purpose: Treatment-related mortality (TRM), defined as death in the absence of progressive cancer, is a major barrier to improved survival in Acute Lymphoblastic Leukemia (ALL) in a low-and-middle country (LMIC) setting. Understanding TRM is crucial to guide supportive care interventions and resource-adapted protocol modifications. This study aimed to determine the prevalence of TRM in children with ALL treated at Philippine Children's Medical Center (PCMC), and also to describe the clinical characteristics, and identify causes of TRM

Methods: A retrospective cross-sectional study on pediatric ALL patients (ages 1-18) treated with modified Berlin Frankfurt Münster 95 /Hong Kong ALL 97 (BFM95/HK ALL97), Children's Cancer Study Group 1961 (CCG 1961), or Augmented BFM regimens was conducted from January 2014 to December 2023. Chart review of mortalities identified from the Microsoft Excel ALL Data Registry was done. Alexander et al.'s TRM classification and cause-of-death attribution system was used to identify TRM. Descriptive statistics summarized demographic, clinical, and treatment data

Results: Among 400 patients, 73 deaths occurred (18.25%). Fifty-one were TRM, yielding an overall prevalence of 12.5%. TRM occurred frequently between ages 1 and 10 years (64.71%), among males (60.78%), in B-cell lineage immunophenotype (76.47%), and high-risk patients (62.7%). Most of the patients had CNS1 status at diagnosis (70.6%) but 21.57% had an unknown CNS status at the time of death. Across treatment protocols, TRM clustered in induction phase of treatment (55.6-83.3%). Infections were the leading causes of death (47.4%).

Conclusion: The overall TRM prevalence was 12.75%, consistent with LMIC reports. Majority of deaths predominantly occurred during induction phase and among high-risk patients. Infections were the leading causes of TRM. Although standard risk B-cell ALL constituted the largest subgroup, high risk patients contributed disproportionately to TRM. Early deaths often limited complete CNS staging. The study provided insights to TRM in pediatric ALL within a local setting.

Abstract 145

Challenging case of a pair of twin presenting acute liver failure as manifestation of secondary hemophagocytic lymphohistiocytosis

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Purpose: Hemophagocytic lymphohistiocytosis (HLH) is a hematological emergency disorder that may mimic other causes, such as sepsis, acute liver failure, and disseminated intravascular coagulation. Acute liver failure as the presenting manifestation of HLH is rare and diagnostically challenging. Reports involving twin patients are exceedingly uncommon.

Methods: We report a challenging referral case of twin girls who presented with acute liver failure. Both patients were admitted with progressive jaundice, high fever, and altered mental status consistent with hepatic encephalopathy; hepatosplenomegaly was also present. There was no parental consanguinity and no family history of liver disease. The clinical presentation of the first twin was more severe than the second; she experienced generalized tonic-clonic seizures, melena, and was intubated at the referral hospital, whereas the second twin remained stable without intubation. Laboratory evaluation for both twins revealed anemia, thrombocytopenia, markedly elevated transaminases, hyperbilirubinemia, coagulopathy, hyperferritinemia, and hyperammonemia. Hepatitis viral markers were negative. Packed red cells and fresh frozen plasma were administered. Due to severe metabolic disturbances, both twins underwent exchange transfusion twice. Following the procedure, the first twin's condition continued to deteriorate, characterized by recurrent melena, hypercapnia, and coma. She passed away after 18 days in the PICU. Conversely, the second twin showed clinical improvement following exchange transfusion, with a decrease in bilirubin, transaminase, and ammonia levels, as well as a return to full alertness. Due to the clinical instability of the first twin, a bone marrow aspiration was only performed on the second twin. The results revealed low granulopoiesis, positive active macrophages, and hemophagocytosis, leading to a diagnosis of HLH. The second twin was discharged and commenced chemotherapy according to the HLH protocol. However, after the third week of chemotherapy, she presented with fever, cough, dyspnea, and vomiting, necessitating readmission. On the second day of admission, she suffered a seizure and rapidly deteriorated. Prior laboratory results prior to her death revealed extreme transaminitis (ALT 27,852 U/L and AST 28,421 U/L) and severe coagulopathy (PT 81.8 seconds; aPTT 69.2 seconds).

Results: Acute liver failure is uncommon presentation of HLH. Delayed recognition resulted to delayed management. The concomitant presentation in twins raises important considerations regarding shared infectious triggers or genetic susceptibility.

Conclusion: Secondary HLH should be considered in children presenting with unexplained acute liver failure. Early recognition and prompt initiation of HLH-specific therapy are crucial to improve outcomes.

Abstract 146

Malignancy associated haemophagocytic lymphohistiocytosis: a battle within a war

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Purpose: Malignancy associated HLH (M-HLH) is a rare potentially life-threatening secondary HLH commonly associated with haematolymphoid malignancies. We present the clinical spectrum of M-HLH diagnosed and treated in our department.

Methods: Data of children diagnosed to have M-HLH associated with leukaemia or lymphoma from 2012- 2024 was retrieved from EMR and analysed.

Results: 19 children, 13 boys and 6 girls, median age 8.7 years were included. Spectrum of malignancies included Hodgkin lymphoma [HL] (4), ALCL (4), SPTCL (4), PTCL-NOS (2) extra nodal NK- T cell lymphoma [ENKTCL] (2) and cutaneous T cell lymphoma [CTCL], B cell ALL and JMML in one each. Median serum ferritin level was 15276ng/ml, 74% had hemophagocytes in the bone marrow, EBV serology was positive in 2. 17 opted for treatment and were started on dexamethasone as per HLH 2004 protocol, in addition to definitive treatment for the underlying malignancies. Details of chemotherapy were as follows: HL- Euronet PHL C1, ALCL - BFM-NHL 95 protocol, SPTCL-CHOP or CHEOP, PTCL- CHOP, CTCL received steroids with methotrexate, ENKTCL- LVP followed by SMILE chemotherapy. Etoposide was added in 5 patients (ENKTCL-2 and one each with PTCL, SPTCL ad HL), cyclosporin in 2 (PTCL and HL), and baricitinib in one as the response to treatment was inadequate. In this cohort where treatment had to be intensified, only 2 are alive. At a median follow up period of 25 months, 10 are in CR1. The causes of death included uncontrolled HLH (6), refractory lymphoma (2) and treatment refusal (2). None of our patients received Ruxolitinib or Emapalumab.

Conclusion: Children with M-HLH in our series had a mortality rate of 47%, at median follow up of 2 years, which is similar to published data. Further studies are required to evaluate if addition of novel therapeutic agents will improve outcome in these children.

Abstract 147

A SINGLE CENTER STUDY ON THE OUTCOME OF PEDIATRIC ACUTE LYMPHOBLASTIC LEUKEMIA WITH 10-YEAR FOLLOW-UP

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Purpose: Oncology Center, Vietnam National Children's Hospital used CCG 1991 and CCG 1961 protocols for over a decade, but no comprehensive evaluation of treatment outcomes has been conducted. Minimal residual disease (MRD)

assessment by flow cytometry at the end of induction (EOI) is considered as one of indications for risk stratification and guiding treatment. The role of MRD at EOI treatment was also evaluated.

Methods: A cohort study included 270 patients newly diagnosed and treated for ALL from January 2016 to December 2020, with 10-year follow-up till August 2025. MRD at the EOI, along with other factors such as immunophenotype, age, initial white blood cell count, karyotype, day 8 bone marrow evaluation and poor prognostic translocations identified by FISH (MLL, TCF3/ PBX1,BCR/ABL) were used to stratify patients into two risk groups (high risk and low risk).

Results: The 5-year event-free survival (EFS) and 10-year overall survival (OS) rates were 66% ± 3% and 50% ± 8%, respectively. Relapse occurred in 20.7% of patients, with three-quarters was bone marrow relapses. The mortality rate due to infections was 8.9%. High-risk patients accounted for 38.9%. However, there was no significant difference in EFS and OS between the high-risk and low-risk groups ($p > 0.05$). In the low-risk group, 5-year EFS and 10-year OS were 65% ± 4% and 49% ± 11%, respectively. In the high-risk group, 5-year EFS and 10-year OS were 62% ± 5% and 54% ± 7%, respectively. Notably, when evaluating MRD at the EOI, patients achieving complete remission (MRD < 0.01%) had a lower 5-year EFS (64% ± 3%) compared to the remaining group (74% ± 7%), although this difference was not statistically significant.

Conclusion: Pediatric ALL outcomes are lower than those in middle-income countries and significantly lower than in developed countries. An updated protocol and better strategy for risk stratification are needed.

Abstract 148

Antibiotic Prophylaxis and Development pf Fever and Infection in Children and Adolescents with Acute Lymphoblastic Leukemia Following Chemotherapy-Induced Neutropenia

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Purpose: Antibiotic prophylaxis is generally not recommended in afebrile chemotherapy-induced neutropenia (CIN) because the potential risks, particularly antimicrobial resistance, may outweigh the benefits of reducing fever and bacteremia. However, its role in low- and middle-income countries remains uncertain. This study evaluated the occurrence of fever and infection among pediatric patients with acute lymphoblastic leukemia (ALL) who developed CIN.

Methods: Medical records of patients aged 1 to <19 years with ALL who developed CIN between January 2017 and December 2022 were retrospectively reviewed. Demographic and clinical data were collected, and patients were categorized according to receipt of antibiotic prophylaxis. Outcomes assessed included development of fever, clinically or microbiologically confirmed infection, and subsequent absolute neutrophil count (ANC). Data were analyzed using descriptive statistics, risk ratios, and Fisher exact test.

Results: A total of 270 patients were included. The mean age was 5.58 ± 3.55 years, with a male-to-female ratio of 2.1:1; most patients had normal nutritional status (72.2%). No significant difference was observed in the likelihood of fever (95% CI 0.14–1.05; p=0.06) or further ANC decline (95% CI 0.60–1.14; p=0.26) between groups. Although the risk of infection appeared lower in the antibiotic prophylaxis group (95% CI 0.27–1.00; p=0.05), Fisher exact test did not demonstrate a significant association.

Conclusion: Antibiotic prophylaxis was not associated with a statistically significant reduction in fever or infection among pediatric ALL patients with CIN. These findings do not support routine prophylactic antibiotic use and underscore the need for larger prospective studies to clarify its role in resource-limited settings.

Abstract 149

Clinical Staging & Treatment Outcomes of Non-Hodgkin Lymphoma: A Single Center Experience from Pakistan

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Purpose: Childhood Non-Hodgkin Lymphoma (NHL) is fourth most common pediatric malignancy but data from low middle-income countries remain scarce. This study evaluates clinical presentation, treatment results and prognostic factors from a tertiary care center in Pakistan with standardized protocols.

Methods: A prospective study was conducted at Pediatric Oncology Department of Combined Military Hospital Rawalpindi, Pakistan from January 2012 to December 2024. Newly diagnosed NHL patients (aged 1-18 years) were treated according to standardized protocols. Demographics, staging, histopathology, treatment response and survival outcomes were analyzed.

Results: Two hundred children with NHL including 156 (78%) boys and 44 (22%) girls were evaluated. The mean age at presentation was 7.44 (IQR 5.0-9.3) years. Burkitt lymphoma (n=118, 58.5%) was the most common tissue diagnosis and abdomen (n=111, 55.5%) being the most commonly involved site. High risk NHL was seen in 165 (82.5%) cases. In univariate analysis, Leucocyte count at diagnosis (p 0.07), hepatomegaly (p 0.05), CNS involvement (p 0.00), stage of disease (p 0.03) significantly affected overall survival (OS) and in multivariate analysis, CNS involvement (p 0.01) was a significant poor prognostic factor. At a median follow up time of 1154 days (IQR 140-2807), OS, DFS and PFS were 59.8%, 66.2% and 59.5% respectively.

Conclusion: Standardized protocols and good supportive care can improve survival outcomes in Non-Hodgkin Lymphoma in Pakistan in comparison to high income countries. CNS involvement was the strongest predictor of poor survival.

Abstract 150

Impaired Branched-chain Amino Acids Catabolism, Marked by BCKDK, Links mTORC1–RRM2 Activation to Immune Suppression in Pediatric AML

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Purpose: Both increased branched-chain amino acid (BCAA) catabolism and reverse flux toward BCAA resynthesis have been linked to tumor progression, highlighting unresolved metabolic heterogeneity. In pediatric acute myeloid leukemia (AML), the flux signatures of BCAA metabolism and their clinical and immunologic relevance remain unclear.

Methods: Using METAflux, we inferred BCAA metabolic flux in pediatric AML from the TARGET cohort and evaluated survival associations. Functional studies were performed by modulating the BCKDH regulatory checkpoint through BCKDK knockdown or overexpression in vitro and in vivo, with rescue experiments using BCAT1 inhibition, mTORC1 inhibition, and BCAA supplementation. Immune features were analyzed by CIBERSORT, while transcriptomic, PPI, single-cell RNA sequencing, and CellChat analyses were used to identify downstream effectors and blast–T-cell communication.

Results: Flux-based clustering identified a previously unrecognized pediatric AML subgroup characterized by elevated BCAA-related gene expression but reduced BCAA catabolic flux, and associated with poor prognosis. BCKDK emerged as a key regulatory checkpoint within the irreversible catabolic pathway. Its modulation altered BCAA retention, mTORC1 activity, and leukemic growth, which were attenuated by BCAT1 or mTORC1 inhibition. Impaired BCAA catabolism was further associated with an immune-cold marrow phenotype, reduced cytotoxic lymphocyte signatures, and suppressed inflammatory and chemokine programs. Notably, these immunosuppressive microenvironmental features were not recapitulated in the TCGA adult AML cohort. Transcriptomic and PPI analyses identified RRM2 as a central proliferative effector. Single-cell analysis revealed a BCKDK–mTORC1hi blast population with restricted BCAA flux, enriched for myeloid suppressive genes including S100A8/A9, and preferentially interacting with T cells through LGALS9- and MIF-mediated immunosuppressive signaling, accompanied by enrichment of Tcm/Tpex-like T-cell programs.

Conclusion: This study identifies a previously unrecognized BCKDK-driven, flux-restricted BCAA metabolic state in pediatric AML, characterized by mTORC1–RRM2-linked proliferation and immunosuppressive blast–T-cell crosstalk. These findings establish BCAA metabolic restriction as a mechanistically relevant metabolic–immune vulnerability in pediatric AML.

Abstract 151

Clinical Characteristics and Outcomes of Human Herpesvirus 6 Encephalitis After Pediatric Allogeneic Hematopoietic Stem Cell Transplantation

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Purpose: Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is an important curative therapy for children with hematological malignancies. However, severe infectious complications such as human herpesvirus 6 (HHV-6) encephalitis may compromise transplant outcomes. Data integrating clinical features, immune status, pathogen spectrum, and prognosis in pediatric patients remain limited.

Methods: We retrospectively analyzed pediatric patients who developed HHV-6 encephalitis after allo-HSCT at the Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences between November 2019 and September 2025. A 1:3 nested case–control study matched by HSCT time was conducted. Clinical characteristics, immune reconstitution parameters, laboratory and neuroimaging findings, metagenomic next-generation sequencing (mNGS) results, treatment, and outcomes were evaluated. Logistic regression identified risk factors, and survival outcomes were analyzed using Kaplan–Meier methods.

Results: HHV-6 encephalitis occurred in 15 of 303 patients (4.95%), with a median onset of 22 days after HSCT. Seizures were the most common manifestation (46.7%), followed by fever and altered mental status (40.0% each). Younger age was independently associated with encephalitis (OR=0.76 95% CI 0.62~0.93, P = 0.007). All patients showed profound lymphopenia with markedly reduced CD4 and CD8 T-cell counts. Cerebrospinal fluid mNGS detected HHV-6 in all cases, whereas conventional PCR was positive in 61.5%. Mixed intracranial infections were identified in 60.0% of patients. The 3-year overall survival was lower in the encephalitis group (70.1% vs 88.4% P = 0.0924), and relapse-free survival was significantly inferior (58.7% vs 83.8% P = 0.0122).

Conclusion: HHV-6 encephalitis is an early and severe complication after pediatric allo-HSCT, characterized by profound immunosuppression and frequent mixed infections. mNGS improves pathogen detection and facilitates early diagnosis. The association with inferior relapse-free survival suggests that intensified surveillance and immune monitoring may help optimize post-transplant outcomes.

Abstract 152

Efficacy and Complications of Immune Checkpoint Inhibitors in Children with Hodgkin Lymphoma

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Purpose: In 10–20% of children with Hodgkin lymphoma (HL), refractory/relapsed (R/R) disease develops, and treatment standards for this group are not defined. Immune checkpoint inhibitors (ICIs) have demonstrated high efficacy in adults, but their safety and effectiveness in children remain insufficiently studied. To evaluate the efficacy and safety of ICIs in children with R/R HL.

Methods: ICIs were administered to 53 children with R/R HL (median age 13 years). Nivolumab (Nivo) was given to 47 (88.7%) patients, pembrolizumab (Pembro) to 5 (9.4%), and alternating therapy to 1 (1.9%). Median number of prior therapy lines before ICI was 2. Nivo doses: 40 mg (n=30; 56.6%), 3 mg/kg (n=17; 32.1%). Pembro dose: 2 mg/kg (n=6; 11.3%). Median number of ICI cycles was 6 (range 2–28). ICI monotherapy was given to 44 (83.0%) patients, combination therapy to 9 (17.0%). Auto-HSCT with BeEAM conditioning including bendamustine was performed in 23 (43.4%) patients.

Results: Overall response to ICI was achieved in 83.0% (n=44): complete response in 54.7% (n=29), partial response in 28.3% (n=15). At a median follow-up of 1.4 years, 7-year overall survival (OS) was 78.2%, progression-free survival (PFS) was 38.2%. In patients who underwent auto-HSCT after ICI, PFS was significantly higher compared to those without transplantation (59.5% vs. 29.3%). Adverse events (AEs) during ICI therapy were recorded in 8 patients (15.1%). Immune-related AEs: endocrinopathies in 3 (5.7%), aseptic meningitis in 1 (1.9%). Grade 3 AEs: neutropenia in 2 (3.8%), tuberculosis reactivation in 1 (1.9%). One patient (1.9%) died from therapy-related complications (secondary HLH due to undiagnosed PID). Engraftment syndrome after auto-HSCT occurred in 26.1% (6/23).

Conclusion: ICIs demonstrate high efficacy in children with R/R HL; however, subsequent consolidation with auto-HSCT is required to achieve durable remission. ICI therapy is associated with a significant risk of severe complications, requiring careful monitoring, timely drug discontinuation, and glucocorticosteroid administration

Abstract 153

The Role and Mechanism of LY86 in Acute Myeloid Leukemia: A Bioinformatics Analysis

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Purpose: Background Acute Myeloid Leukemia (AML) is a highly aggressive hematologic malignancy and a leading cause of adult leukemia-related deaths. Despite advances in therapy, patient outcomes remain poor, particularly for those with adverse-risk genetics or who experience relapse, highlighting an urgent need for novel diagnostic and prognostic biomarkers as well as therapeutic targets. The tumor immune microenvironment plays a critical yet complex role in AML progression and treatment response. Lymphocyte Antigen 86 (LY86), also known as MD-1, is a co-receptor involved in modulating innate immune responses, particularly within the Toll-like receptor 4 (TLR4) signaling pathway. While its role has been characterized in inflammation and certain solid tumors, its expression pattern, clinical significance, and biological function within the AML immune landscape remain largely unexplored. Preliminary bioinformatics analyses indicate that LY86 is significantly overexpressed in AML and correlates with adverse clinical features, suggesting it may be a key regulator of leukemogenesis and immune evasion. Objectives This study aims to comprehensively investigate the clinical and biological significance of LY86 in AML. Our specific objectives are: 1.To validate the differential expression of LY86 in AML patient samples compared to healthy controls and to assess its diagnostic potential. 2.To evaluate the association between LY86 expression levels and key clinicopathological features, including FAB subtype, cytogenetic risk, and survival outcomes, to determine its prognostic value. 3.To construct and validate a robust prognostic nomogram incorporating LY86 expression and other independent risk factors for personalized survival prediction. 4.To elucidate the potential biological mechanisms and signaling pathways (e.g., Toll-like receptor pathway) through which LY86 may contribute to AML pathogenesis using functional enrichment analysis. 5.To characterize the relationship between LY86 expression and the composition of the immune tumor microenvironment in AML, specifically its correlation with infiltrating immune cell subsets such as dendritic cells, T cells, and macrophages. By achieving these objectives, this research seeks to establish LY86 as a novel multi-functional biomarker for AML, providing insights into its role in disease progression and the immune microenvironment, thereby informing the development of more precise prognostic tools and potential immunomodulatory therapeutic strategies.

Methods: A pan cancer expression profile analysis was conducted based on bioinformatics. AML transcriptomic data from the TCGA, TARGET, and GEO databases (GSE24395, GSE17054) were integrated. Differential expression analysis combined with prognostic correlation screening identified LY86 and 10 other core genes. qRT PCR was used to validate LY86 mRNA expression in bone marrow samples from AML patients. Based on expression levels, the TCGA AML cohort was divided into high and low expression groups to analyze their correlations with clinicopathological features and prognosis. A Cox proportional hazards model was employed to construct a prognostic nomogram. GO/KEGG enrichment analyses were performed to elucidate biological pathways, and the CIBERSORT algorithm was applied to assess immune infiltration characteristics.

Results: Pan cancer expression profiling showed that LY86 exhibited significantly differential expression across 33 malignancies ($p<0.05$), with specific high expression in AML ($p<0.001$). qRT PCR validation confirmed that LY86 mRNA expression was significantly higher in AML patient bone marrow samples

than in healthy controls (p<0.05). Its diagnostic performance was excellent according to ROC curve analysis (AUC=0.99, 95% CI 0.997 1.00). Further analysis revealed that high LY86 expression was significantly associated with elevated white blood cell count (p=0.003), poor prognosis group (p=0.017), and FAB subtypes M4/M5 (p=0.008). Survival analysis in the TARGET cohort showed that patients with high LY86 expression had significantly shorter overall survival (HR=3.39, 95% CI 2.37 4.85), a result independently validated in the TCGA cohort (HR=1.67, 95% CI 1.09 2.56). Univariate analysis identified age >60 years (HR=3.321, p<0.001), intermediate prognosis (HR=2.934, p=0.002), poor prognosis (HR=4.124, p<0.001), and LY86 expression level (HR=1.618, p=0.027) as risk factors affecting AML overall survival (OS). Multivariate Cox regression further confirmed age >60 years (HR=2.884, p<0.001), intermediate prognosis (HR=2.238, p=0.023), poor prognosis (HR=2.896, p=0.008), and LY86 expression (HR=1.706, p=0.020) as independent risk factors for AML prognosis. An AML prognostic nomogram model was constructed. The model demonstrated stable predictive performance in the training set (1/3/5 year AUC: 0.82/0.79/0.76). Calibration curves indicated high agreement between predicted and observed survival rates (C index=0.85; Hosmer–Lemeshow test p>0.05), and decision curve analysis (DCA) showed clinical net benefit over a wide threshold range. Functional enrichment analysis revealed that LY86 participates in disease progression by regulating Toll like receptor signaling pathways and immune receptor activation. Moreover, immune microenvironment analysis based on the CIBERSORT algorithm showed that LY86 expression was significantly positively correlated with infiltration levels of immature dendritic cells (r=0.652, p<0.01), memory T cells (r=0.558, p<0.01), and macrophages (r=0.541, p<0.01), suggesting that LY86 may influence AML pathogenesis by reshaping the immune microenvironment.

Conclusion: LY86 serves as a novel molecular marker for AML, combining diagnostic specificity (AUC=0.99), prognostic predictive efficacy (HR=1.71), and immune microenvironment regulatory functions, providing a theoretical basis for precision treatment strategies.

Abstract 154

POLD1 Drives B-Cell Non-Hodgkin Lymphoma Progression Through RLR Signaling and Represents a Potential Therapeutic Target

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Purpose: Relapsed or refractory diffuse large B-cell lymphoma (DLBCL) remains a major clinical challenge, particularly in children and adolescents, who have limited treatment options and poor outcomes after relapse. Increasing evidence indicates that dysregulated tumor-intrinsic signaling contributes to lymphoma progression and therapeutic resistance, but the key upstream drivers are not fully understood. Our preliminary studies identified DNA polymerase delta 1 (POLD1) as aberrantly overexpressed in B-cell non-Hodgkin lymphoma (B-NHL), where it is associated with malignant progression and unfavorable prognosis. This study aimed to investigate the biological role of POLD1 in B-NHL/DLBCL, explore its relationship with RIG-I-like receptor (RLR) signaling, and evaluate its potential as a therapeutic target.

Methods: Public RNA-seq data, clinical samples, cell-based assays, and xenograft models were used to study POLD1 in B-NHL/DLBCL. POLD1 knockdown, multi-omics profiling, poly(I:C) rescue, and resveratrol treatment were applied to explore its function, downstream signaling, and therapeutic potential.

Results: POLD1 was significantly overexpressed in DLBCL cell lines and pediatric relapsed/refractory tumor samples, and correlated with advanced stage, high-risk stratification, and poor survival. POLD1 knockdown suppressed proliferation, induced S-phase arrest and apoptosis, and significantly inhibited tumor growth in vivo. Multi-omics analysis identified the RLR pathway as a key downstream signaling axis of POLD1. Poly(I:C) partially rescued the growth inhibition caused by POLD1 knockdown. Moreover, resveratrol markedly increased apoptosis and inhibited proliferation of Raji cells in a dose-dependent manner. Western blot analysis showed that resveratrol reduced POLD1, TRAF3, and IRF3 expression while increasing Caspase-3 levels, suggesting that resveratrol suppresses B-NHL growth at least partly through the POLD1/RLR axis.

Conclusion: POLD1 is a potential prognostic biomarker and functional driver in B-NHL/DLBCL. Targeting the POLD1-associated RLR pathway, including by resveratrol-mediated POLD1 suppression, may offer a promising therapeutic strategy for relapsed/refractory pediatric lymphoma.

Abstract 155

LILRB3 Supports the Development of Acute Lymphoblastic Leukemia and Regulates NK Cell Anti-tumor Immune Responses via the LILRB2–Erk Signaling Axis

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Purpose: Acute B-lymphoblastic leukemia (B-ALL) is a common and highly fatal malignancy, with mortality rates remaining particularly high in patients with relapsed/refractory disease. Biomarkers closely associated with its prognosis are still under intensive investigation. This study aimed to integrate bioinformatics analysis with functional studies of target genes in B-ALL to identify potential molecular markers with clinical applicability and prognostic significance, thereby offering new insights for prognostic assessment and targeted therapy.

Methods: Utilizing primary cells from B-ALL patients and corresponding single-cell RNA sequencing (scRNA-seq) data, we employed computational bioinformatics methods for biomarker screening and validation. Subsequent in vitro functional assays were performed in Nalm-6 and Ball-1 B-ALL cell lines following Tmem263 knockdown via RNA interference, including assessments of cell proliferation (CCK-8 assay), apoptosis and cell cycle distribution (flow cytometry), and immune evasion capacity (CFSE staining). In vivo leukemia progression was evaluated in a B-ALL mouse model. Transcriptomic analysis was conducted on TMEM263-knockdown cells to explore downstream mechanisms.

Results: Our bioinformatics analysis identified transmembrane protein 263 (Tmem263), a member of the transmembrane protein family (Tmems), as significantly upregulated in B-ALL patient samples compared to normal controls. Tmem263 expression levels were negatively correlated with tumor purity and the proportion of tumor-infiltrating immune cells, suggesting a potential role in immune microenvironment regulation. Functionally, Tmem263 knockdown significantly inhibited cell proliferation, induced cell cycle arrest, and enhanced NK cell activation in vitro. In the B-ALL mouse model, Tmem263 knockdown effectively delayed leukemia progression and markedly reduced tumor infiltration in the liver and spleen. Transcriptomic profiling of TMEM263-knockdown cells revealed increased expression and transcriptional activity of LILRB2, alongside elevated Erk expression, corroborating these findings. Importantly, blocking the LILRB2 signaling pathway with a specific antagonist antibody effectively suppressed ALL progression.

Conclusion: This study is the first to systematically elucidate the expression pattern, biological function, and prognostic significance of Tmem263 in B-ALL. Our findings demonstrate that Tmem263 plays a critical oncogenic role in disease progression, potentially via modulation of the immune microenvironment and the LILRB2 pathway. Targeting the Tmem263/LILRB2 axis may represent a promising therapeutic strategy for B-ALL.

Abstract 156

Donor Lymphocyte Infusion for Post-Transplant Relapse in Pediatric BCR-ABL p190-Positive B-Cell Acute Lymphoblastic Leukemia: First Experience in Indonesia

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Purpose: Relapse after allogeneic hematopoietic stem cell transplantation (HSCT) in pediatric Philadelphia chromosome–positive (Ph+) acute lymphoblastic leukemia (ALL) is associated with extremely poor outcomes and limited therapeutic options, particularly in resource-limited settings. Donor lymphocyte infusion (DLI) can induce a graft-versus-leukemia (GvL) effect and may be administered for prophylactic, pre-emptive or therapeutic purposes. However data in children, particularly in low- and middle-income countries, remain scarce. We report the first use of repeated DLI for post-hematopoietic stem cell transplantation (HSCT) relapse at our center.

Methods: An 8-year-old boy with B-cell ALL harboring BCR-ABL p190 achieved initial remission and underwent allogeneic HSCT from a matched sibling donor. He subsequently developed one hematologic relapses and one CNS relapse after transplantation. Following the second relapse, an immunologic salvage strategy was pursued. Immunosuppression was discontinued and two escalating doses of DLI from the original donor were administered. A third-generation tyrosine kinase inhibitor therapy was continued concurrently. Disease status was monitored through bone marrow examination, minimal residual disease (MRD), and BCR-ABL transcript levels, while graft-versus-host disease (GVHD) and treatment-related toxicity were closely observed.

Results: The first DLI was associated with a reduction in leukemic burden, suggesting a clinically meaningful GvL response. A second DLI was administered without significant infusion-related toxicity or severe acute GVHD. However, bone marrow evaluation a week after the DLI demonstrated recurrent leukemic relapse, indicating a transient and non-durable response.

Conclusion: Repeated DLI is feasible and can provide effective immunologic disease control in pediatric patients with Philadelphia chromosome positive ALL relapsing after HSCT. This represents the first reported experience at our institution and, to our knowledge, in Indonesia. Our findings support the incorporation of DLI into relapse management strategies in resource-limited settings and highlight the need for collaborative prospective studies.

Abstract 157

VINCRISTINE-INDUCED NEUROPATHY IN PEDIATRIC ACUTE LYMPHOBLASTIC LEUKAEMIA

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Purpose: Vincristine-induced neuropathy (VIN) is a common adverse effect of vincristine therapy in pediatric acute lymphoblastic leukaemia (ALL). Early detection is important to reduce neurological complications and maintain effective chemotherapy treatment. This study aimed to determine the prevalence and severity of VIN and its association with clinico- demographic characteristics.

Methods: A hospital based prospective descriptive study was conducted in the Haematology and Oncology Ward of Mandalay Children's Hospital, Myanmar, from October two thousand twenty four to October two thousand twenty five. Fifty four newly diagnosed pediatric patients with ALL aged two to twelve years were enrolled. Patients were assessed weekly from day one to day fifty six for neuropathy signs and symptoms. Severity was evaluated using the Total Neuropathy Score-Pediatric Vincristine (TNS-PV), and data were analyzed using STATA version fifteen point one with the chi square test.

Results: Among fifty four pediatric patients with acute lymphoblastic leukaemia, most were males aged five to eight years with normal nutritional status. Vincristine-induced neuropathy occurred in twenty five point nine percent of patients, and most cases were mild with sensory neuropathy predominating. A higher occurrence of VIN was observed among patients aged nine to twelve years, females, those weighing more than ten kilograms, and high risk patients. The mean cumulative vincristine dose was five point one nine plus minus one point three four milligrams, with no statistically significant association with clinico-demographic characteristics.

Conclusion: Vincristine-induced neuropathy occurred in about one quarter of pediatric ALL patients and most cases were mild. Early monitoring may improve patient safety and treatment outcomes. Larger multicenter studies are recommended to evaluate long term outcomes of VIN in pediatric ALL patients. The authors sincerely acknowledge the University of Pharmacy Mandalay, Mandalay Children's Hospital, supervisors, and colleagues for their guidance and support in this study.

Abstract 158

A multivariate analysis of Survival Outcomes and Prognostic Factors in Childhood Hodgkin Lymphoma

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Purpose: Childhood Hodgkin lymphoma (HL) accounts for 5% of pediatric malignancies, but data from low-middle-income countries (LMICs) on patient characteristics and outcomes remain scarce. This study evaluates clinical presentation, treatment results, and prognostic factors in a Pakistani cohort treated with standardized protocols.

Methods: A prospective study was conducted at the Pediatric Oncology Department of Combined Military Hospital Rawalpindi, Pakistan from January 2012 to December 2024. Newly diagnosed HL patients (aged 1–15 years) were treated per the EuroNet-PHL-C1 trial's standard arm (OEPA and COPDAC chemotherapy ± involved-field radiotherapy). Demographics, staging, histopathology, treatment response, and survival outcomes were analyzed.

Results: Among 221 patients (76.9% male, mean age 7.0±2.99 years), cervical lymphadenopathy (83.1%) and B symptoms (48.9%) were common. Stage of the disease was stage I (13.8%), II (28.9%), III (40.8%), IV (16.5%). Mixed cellularity subtype predominated (75.1%). Poor early responders (8.6%) received radiotherapy. Univariate analysis identified delayed oncologist referral (P=0.001), bone marrow involvement (P=0.062), elevated LDH (P=0.058), and poor nutritional status (P=0.026) as adverse prognostic factors; delayed referral remained significant in multivariate analysis (P=0.08). At median follow-up of 12.5 years, 5-year overall survival (OS) and disease-free survival (DFS) were 92.3% and 91.4%, respectively.

Conclusion: Childhood HL in Pakistan achieves outcomes comparable to high-income countries when treated with standardized protocols. Delayed referral to pediatric oncologists was the strongest predictor of inferior survival, underscoring the need for public awareness and early diagnosis in LMICs.

Abstract 159

Multiparameter flow cytometry versus droplet digital PCR for monitoring measurable residual disease in pediatric AML

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Purpose: Measurable residual disease (MRD) monitoring is crucial for prognostic stratification in pediatric acute myeloid leukemia (AML).

Methods: This study compared the performance of multiparameter flow cytometry (MFC) and droplet digital PCR (ddPCR) for MRD assessment in 116 pediatric AML patients at end of induction (EOI)-1, EOI-2 and end of consolidation (EOC)-1.

Results: At EOC-1, MFC detected MRD < 0.01% in 87.1% of patients, compared to 50.9% by ddPCR. MRD clearance is significantly delayed when assessed by ddPCR compared to MFC in all genetic subtypes of AML.

Conclusion: Concordance between MFC and ddPCR was only moderate. Both MFC and ddPCR were significant predictors of 5-year event-free survival (EFS) at EOIs. For instance, MFC-MRD ≥1% at EOI-1 was associated with a 5-year EFS of 50.2 ± 19.8% vs. 79.4 ± 6.4% for patients with MRD <1% (P= 0.003), while the survival rates for ddPCR groups (≥1% and <1%) were 41.0 ± 23.8% vs. 91.7 ± 4.5% (P < 0.001). Notably, the prognostic power of MFC diminished at EOC-1, while ddPCR remained significant. Subgroup analysis by genetic alterations revealed that ddPCR provided significant prognostic stratification across all genetic subgroups while MFC did not. When ddPCR-MRD <1% at EOI-1 or <0.1% at EOI-2, the MFC status has minimal impact on prognosis, and combining MFC and ddPCR results created a more powerful risk model. In conclusion, ddPCR demonstrates superior sensitivity and sustained prognostic value over MFC across all genetic subtypes in pediatric AML, and integrating the two methods enables more precise risk stratification.

Abstract 160

Tyrosine kinase inhibitors therapy in children with diffuse cutaneous and aggressive systemic forms of mastocytosis

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Purpose: Pediatric mastocytosis is a heterogeneous group of the diseases characterized by accumulation of clonal mast cells in different organs. Cutaneous mastocytosis with benign course is the most common form in children. Despite on spontaneous regression in some cases diffuse unregressing mastocytosis is observed. This one and a systemic form of mastocytosis may be treated with tyrosine kinase inhibitors, depending on KIT mutation status.

Methods: From 2023 to 2026, 10 patients (6 (60%) boys, 4 girls (40%)) were included in our study: 9 (90%) – with diffuse cutaneous mastocytosis; 1 (10%) – with aggressive systemic form. The median age of patients was 14 month (0 – 156). Tryptase level less than 20 ng/ml was observed in 4 (40%) patients, 20 – 100 ng/ml – in 2 (20%) patients, more than 100 ng/ml – in 1 (10%) patient (with systemic form). In 3 (30%) cases the tryptase level was unknown. One patient with diffuse cutaneous mastocytosis without KIT mutation was treated with imatinib, 2 patients with KIT D816Y and D816V mutations – with midostaurine. The patient with aggressive systemic form and KIT D419del mutation was treated with imatinib. Topical and systemic corticosteroids were prescribed in other cases. The treatment associated toxicity was not observed.

Results: As a result of therapy with imatinib partial response was achieved in patient with aggressive systemic form. Unfortunately, disease progression was diagnosed after 7 month from start therapy that caused to death. Patient with diffuse cutaneous mastocytosis who was treated with imatinib had complete response within 20 months. Treatment with midostaurine resulted in marked improvement in two patients. One of them had progression after 7 months of the treatment. Patients who treated with corticosteroids achieved significant improvement in the healing of skin lesions.

Conclusion: Tyrosine kinase inhibitors may be possible treatment option in children with diffuse cutaneous and systemic mastocytosis.

Abstract 161

CD45RA-targeted CAR-T therapy for acute myeloid leukemia: preclinical evaluation and optimization

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Purpose: Acute myeloid leukemia (AML) lacks an ideal CAR-T target because current candidates such as CD33, CLL-1, and CD123 show heterogeneous expression, limited leukemic coverage, and potential off-tumor toxicity. We evaluated CD45RA as a novel target and optimized CD45RA-directed CAR design.

Methods: We integrated public bulk and single-cell datasets, performed flow cytometry in AML cell lines, pediatric primary samples, and healthy HSPCs, generated 3A4-based CD45RA CAR-T cells, and assessed them by in vitro assays, xenografts, dose testing, scFv orientation comparison, and RNA-seq.

Results: CD45RA was the predominant AML CD45 isoform, with lower expression in early HSPCs than pan-CD45. 3A4 recognized AML cell lines and pediatric primary samples. CD45RA CAR-T cells were active, and the 28z-1XX design showed the best dose-dependent in vivo control with a less exhausted profile.

Conclusion: CD45RA shows high expression, broad leukemic coverage, and a degree of isoform selectivity in AML, supporting its potential as a novel CAR-T target. CD45RA-targeted CAR-T cells generated from the 3A4-derived scFv demonstrated effective anti-leukemic activity in vitro and in vivo, with the CD45RA-28z-1XX configuration showing the best overall therapeutic performance. These findings support the feasibility of CD45RA as a therapeutic target in AML and provide a rationale for further optimization of CD45RA-directed CAR design.

Abstract 162

Emapalumab in pediatric relapsed/refractory hemophagocytic lymphohistiocytosis: clinical responses, attenuated efficacy upon retreatment, and role in bridging to HSCT

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Purpose: R/R HLH represents a hyperinflammatory state driven largely by IFN-γ dysregulation and carries a high risk of early mortality and failure to bridge to HSCT. Emapalumab has emerged as a targeted option, but evidence in heterogeneous real-world pediatric populations remains limited.

Methods: We retrospectively reviewed pediatric patients with R/R HLH who received emapalumab at our center from April 2023 to July 2025. Clinical responses, cytokine kinetics, virologic parameters, survival outcomes, and HSCT feasibility were assessed.

Results: Ten children were included: primary HLH (n=3), EBV-HLH (n=5), and malignancy-associated HLH (n=2). Early clinical improvement was observed in most patients, with an ORR of 70% (3 pHLH, 3 EBV-HLH, and 1 M-HLH). IFN-γ, IL-6 and CXCL9 levels declined markedly among responders.

Conclusion: Five (3 pHLH and 2 EBV-HLH) successfully proceeded to HSCT, four of whom achieved sustained remission. Patients requiring a second course of emapalumab exhibited attenuated fever resolution and reduced cytokine clearance. During a median follow-up of 3.8 months, the 6-month OS was 45.3 ± 29.2%. Five patients (3 pHLH and 2 EBV-HLH) remained alive at the time of last follow-up. No newly emerged treatment-related toxicities were observed. Emapalumab rapidly controls hyperinflammation in pediatric R/R HLH and enables HSCT in a substantial subset of patients. Diminished responsiveness during retreatment underscores the need for timely disease-modifying therapy. Integrated evidence supports IFN-γ blockade as a key component of multimodal HLH management.

Abstract 163

Multisystem Langerhans Cell Histiocytosis in a Child with Noonan Syndrome: A Case Highlighting Shared MAPK Pathway Dysregulation

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Purpose: Langerhans cell histiocytosis (LCH) is a histiocytic disorder caused by MAPK pathway activation, often due to somatic BRAF mutations. Noonan syndrome is a congenital RASopathy affecting the same pathway. We report a pediatric case of multisystem LCH associated with Noonan syndrome.

Methods: Clinical, radiological, pathological, and genetic findings were evaluated in a child diagnosed with multisystem LCH. Molecular analysis of tumor tissue and whole exome sequencing were performed as part of the diagnostic evaluation.

Results: A 1.5-year-old child was diagnosed with multisystem LCH involving the skin and bones, with risk organ involvement of the liver and spleen. The patient presented with severe growth retardation, developmental delay, pulmonary artery stenosis, and dysmorphic features including down-slanting palpebral fissures, low-set ears, and a short neck. Histopathological examination confirmed LCH, and molecular analysis of the lesion detected a BRAF V600E mutation. Treatment was initiated according to the LCH-IV protocol. After the first six weeks of induction therapy, the patient developed septic shock and a fungal phlegmon requiring intensive care management. PET-CT performed after the initial course of therapy demonstrated a partial response. Due to the presence of syndromic features and significant treatment toxicity, whole exome sequencing was performed and identified a heterozygous pathogenic PTPN11 variant consistent with Noonan syndrome. Therapy was subsequently changed to targeted treatment with the BRAF inhibitor dabrafenib. The patient demonstrated clinical improvement with weight gain and currently remains in remission.

Conclusion: This case highlights the rare coexistence of multisystem LCH and Noonan syndrome and underscores the importance of genetic evaluation in patients with syndromic features. Identification of MAPK pathway alterations may guide the use of targeted therapies in selected patients.

Abstract 164

Temporal Bone and Calvarial Langerhans Cell Histiocytosis Mimicking Osteomyelitis in a Two-Year-Old Child: A Diagnostic Pitfall

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Purpose: Langerhans cell histiocytosis (LCH), a rare clonal disorder of myeloid dendritic cells, commonly affects the pediatric skeleton. Calvarial involvement may present with otorrhea and scalp fluctuance, mimicking complicated otomastoiditis. Such presentations may delay diagnosis and proper management.

Methods: A 2-year old boy presented with right parietal and temporal scalp masses and serosanguinous otorrhea. CT revealed two calvarial bone erosive lesions. He was treated as otomastoiditis with osteomyelitis and epidural abscess. He received 43 days of antibiotics prior to referral for extensive surgery.

Results: Reassessment revealed clinical absence of systemic infection. CT showed multifocal lytic calvarial lesions with beveled margins, labyrinth-sparing, suggestive of LCH. This allowed cancellation of planned cranial and otologic surgery, and biopsy was instead done, which confirmed the diagnosis.

Conclusion: LCH should be considered in children with destructive calvarial lesions, especially if multifocal, labyrinth-sparing and with beveled edges. Independent reassessment of clinical data and recognition of key radiologic features are essential to prevent misdiagnosis and unnecessary radical procedures.

Abstract 165

Atorvastatin Targeting ITGB2 Combined with Venetoclax Regulates Immunogenic Cell Death and the Immune Microenvironment in AML

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Purpose: AML is an aggressive hematologic malignancy marked by immune evasion and therapeutic resistance. ICD may overcome immune escape, but its regulation in AML remains unclear. We investigated whether ITGB2 drives immune evasion and whether its targeting enhances ICD and venetoclax sensitivity.

Methods: AML bone marrow scRNA-seq was analyzed using an ICD signature to identify regulators, with ITGB2 prioritized by co-expression and Cox analyses. ITGB2-knockdown models, transcriptomics, cell-cell interaction analysis, and virtual screening were used to assess mechanism and venetoclax synergy.

Results: ITGB2 was highly expressed in AML and correlated with poor prognosis. ITGB2 knockdown promoted apoptosis, pyroptosis, and DAMP release through the PDE4A/cAMP/NF-κB axis, while also enhancing sensitivity to venetoclax. Atorvastatin exerted similar anti-leukemic effects.

Conclusion: ITGB2 promotes AML immune escape by restraining apoptosis/pyroptosis-driven ICD and by suppressing DC-mediated immune activation within the leukemic microenvironment. Targeting ITGB2 with atorvastatin restores immunogenic cell death, remodels the immune microenvironment, and sensitizes AML cells to venetoclax. These findings identify ITGB2 as a promising therapeutic target and support a rationale for combining ITGB2-targeted strategies with venetoclax in AML.

Abstract 166

First Experience of Autologous Hematopoietic Stem Cell Transplantation in Patients with Hodgkin Lymphoma at the Rostov Regional Children's Clinical Hospital

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Purpose: Hodgkin lymphoma (HL) often affects adolescents and young adults. Although first-line therapy achieves complete remission in most cases, 5–15% of patients remain refractory. For them, high-dose chemotherapy followed by autologous stem cell transplantation is the treatment of choice.

Methods: Two depersonalized cases of patients with refractory HL who underwent HDCT with BEAM conditioning followed by auto-HSCT were retrospectively analyzed.

Results: Two 14-year-old patients with refractory Hodgkin lymphoma achieved complete remission after second-line therapy (IGEV or pembrolizumab-based), followed by BEAM high-dose chemotherapy and autologous stem cell transplantation, with manageable post-transplant complications.

Conclusion: HDCT followed by auto-HSCT is an effective therapeutic option for pediatric patients with refractory HL achieving CMR after second-line therapy.

Abstract 167

Burkitt Lymphoma in Children and Adolescents

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Purpose: Histopathology combined with immunohistochemistry (IHC) plays a critical role in establishing the diagnosis of Burkitt lymphoma. This multicenter study aimed to characterize the clinicopathological and immunophenotypic features of pediatric and adolescent BL in southern Vietnam.

Methods: We conducted a retrospective descriptive case series of 35 patients diagnosed with BL between January 2020 and December 2024 at three tertiary centers in Ho Chi Minh City, including a major pediatric hospital. Clinical data, histopathological findings, and IHC profiles were analyzed.

Results: All cases showed a germinal center B-cell profile: CD20 /CD10 , negative for CD3, CD5, Cyclin D1, BCL2, CD34, and TdT. BCL6 expression was observed in 90.9%, while most cases were MUM1-negative. All cases demonstrated very high proliferative activity (Ki-67 >95%) and strong c-MYC expression (≥80%).

Conclusion: In settings where molecular testing is not routinely available, a standardized IHC panel combined with classic histomorphology provides a reliable diagnostic approach.

Abstract 168

Early Molecular Kinetics Predict Deep Molecular Response and Inform Frontline TKI Selection in Pediatric Chronic Myeloid Leukemia: A Real-World Multicenter Study

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Purpose: This study aimed to evaluate the ELTS score and early molecular response (EMR, defined as BCR::ABL1 level ≤10% at 3 months) in a real-world pediatric chronic myeloid leukemia (CML) cohort and compare frontline imatinib (IM) versus 2G-TKIs.

Methods: This multicenter, retrospective study analyzed 248 pediatric CML-CP patients from 13 Chinese hospitals. The majority (89.9%) received first-line imatinib therapy. The impact of prognostic factors was evaluated using Kaplan-Meier and Cox regression analyses.

Results: The EMR group achieved MMR and DMR significantly faster and higher. Achieving EMR was independent predictors of MMR and DMR. Front-line 2G-TKIs led to a significantly faster and higher to achieving DMR compared to IM. Baseline ELTS risk stratification showed no prognostic value.

Conclusion: In pediatric CML, EMR are strong predictors of long-term outcomes. Frontline 2G-TKIs may offer a significant benefit over IM for DMR. The prognostic value of the ELTS score in pediatric CML needs further assessment.

Abstract 169

ENDOCRINE SYSTEM INVOLVEMENT IN LANGERHANS CELL HISTIOCYTOSIS

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Purpose: Langerhans cell histiocytosis (LCH) may involve endocrine organs causing dysfunctions (ED) at diagnosis, during reactivation or as late effect. Diabetes insipidus and growth hormone deficiency are the most frequently reported disorders. Endocrine involvement in LCH was evaluated in this study

Methods: The findings of endocrinological dysfunction and clinical characteristics of patients with newly diagnosed LCH at our center between 1972 and 2024 were evaluated retrospectively.

Results: Endocrine dysfunction was noted in 59 (16%) patients. Diabetes insipidus (97%), central hypothyroidism (29%), and growth hormone deficiency (12%) were noted at diagnosis (78%) or reactivation (22%). Symptom duration

was 13.8mo (vs 3.6mo, p<0.0001). Persistent sequelae were noted in 86% of patients.

Conclusion: Endocrine system involvement is not seldom in LCH. The most frequent endocrine dysfunction is diabetes insipidus due to pituitary involvement. Sequelae are permanent in most cases, especially in patients with long duration of symptoms. Patients with LCH should be carefully evaluated for endocrine findings both at diagnosis and during follow-up.

Abstract 170

CLINICAL PROFILE AND TREATMENT OUTCOMES OF PEDIATRIC CHRONIC MYELOGENOUS LEUKEMIA IN A RESOURCE-LIMITED SETTING: A 6-YEAR RETROSPECTIVE STUDY

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Purpose: Pediatric Chronic Myelogenous Leukemia (CML) is rare, accounting for 2% - 3% of pediatric leukemia cases globally. Tyrosine kinase inhibitors (TKIs), such as imatinib, have improved outcomes; however, data on Filipino pediatric patients remain limited.

Methods: Retrospective cohort study of pediatric CML patients (1–18 years) treated with imatinib at the Philippine General Hospital (2019–2024). Demographic, clinical, and laboratory data were reviewed. Treatment response was assessed by CHR, MCyR, MMR, and overall survival.

Results: Thirty-five pediatric CML patients were included, mostly adolescents diagnosed in chronic phase. CHR occurred in 88.2%, MCyR in 18.5%, and MMR in 51.4%. CHR was associated with age >10 years, lower BMI, and fewer peripheral blasts. Overall survival was 71.4%, with notable loss to follow-up.

Conclusion: Imatinib is effective in inducing hematologic remission in pediatric CML. Enhancing access to molecular testing, second-line therapies, and supportive care is essential to improving outcomes in low-resource settings. in this population.

Abstract 171

PREVALENCE OF OVERWEIGHT, OBESITY AND METABOLIC SYNDROME AMONG ACUTE LYMPHOBLASTIC LEUKEMIA SURVIVORS IN A TERTIARY HOSPITAL IN QUEZON CITY, PHILIPPINES

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Purpose: Childhood cancer survivors are at risk for metabolic complications such as obesity, dyslipidemia, insulin resistance, hypertension, and cardiovascular disease. This study assessed overweight/obesity and metabolic syndrome among pediatric ALL survivors.

Methods: The nutritional status of childhood ALL survivors who completed therapy between 2011–2021 at a tertiary hospital in Quezon City was evaluated. Overweight/obesity were determined using BMI, and metabolic syndrome was assessed using IDF criteria with statistical analysis.

Results: Ninety-four patients were included, with 21.2% (n=20) overweight or obese. Metabolic syndrome prevalence in this group was 41%, higher than in the general Filipino population. BMI significantly increased from diagnosis to post-treatment and follow-up (p=0.021, 0.004).

Conclusion: Overweight and obese pediatric ALL survivors have a higher prevalence of metabolic syndrome compared with the general population. Findings suggest continued weight gain during and after treatment, highlighting the need for weight control and lifestyle interventions to reduce cardiovascular risk.

Abstract 172

Targeting SHP2 in immune cells reshapes anti-tumor immunity

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Purpose: SHP2 is a key regulator of MAPK signaling and mediates downstream of the immune checkpoint receptor PD-1 in immune cells. However, its role in immune regulation remains unclear. We evaluated SHP2 inhibition in immune cell models to determine its impact on immune activation and anti-tumor immunity.

Methods: Jurkat, THP-1, and primary immune cells were used to assess immune responses. Activation and differentiation were analyzed by flow cytometry, viability by WST-1. Cytotoxicity in co-culture was measured by LDH assay. In vivo, MC38-OVA tumor-bearing mice received oral TNO155 (10 or 20 mg/kg, BID).

Results: TNO155, a SHP2 inhibitor, did not affect cell viability but suppressed MAPK signaling. SHP2 blockade increased CD69 and Granzyme B, enhancing tumor cell killing (DMSO 8.98% vs TNO155 12.15%, P<0.001). In vivo, TNO155 inhibited tumor growth and increased Granzyme B in tumor-infiltrating lymphocytes.

Conclusion: This study demonstrates the immunomodulatory roles of SHP2 inhibition and reveals the therapeutic potential of targeting SHP2, providing preclinical evidence to support the development of SHP2-directed cancer immunotherapy.

Abstract 173

Beyond Fusion Status: Genomic Predictors of Relapse in Chinese Pediatric AML

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Purpose: Pediatric AML is genetically heterogeneous. Population-specific mutation patterns and their prognostic impact remain incompletely defined.

Methods: Targeted NGS was performed in 100 newly diagnosed pediatric AML patients without prior cytotoxic therapy to characterize mutation profiles and correlate genomic alterations with clinical outcomes.

Results: Mutations were detected in 88% of cases (mean 1.9/patient). NRAS (25%), FLT3-ITD (15%) and KRAS (15%) were most frequent. NUP98-r, IDH2 and FLT3-ITD predicted higher relapse, while AML-ETO showed superior survival.

Conclusion: Distinct mutation patterns in Chinese pediatric AML are linked to outcome heterogeneity.

Abstract 174

Structure-Guided Precision Therapy in TTMV::RARA Pediatric APL

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Purpose: TTMV::RARA is a rare viral–human fusion driving pediatric APL with variable response to standard ATRA+ATO therapy. Optimal treatment remains undefined.

Methods: We analyzed one TTMV::RARA APL case alongside 31 reported cases. AlphaFold2 predicted fusion structure, and primary leukemic cells underwent in-vitro drug sensitivity testing to guide therapy selection.

Results: Modeling identified an arsenic-binding motif (C55/C57/C59), suggesting ATO sensitivity. Functional assays confirmed sensitivity to ATO and venetoclax. A structure-guided ATRA+ATO+VEN regimen achieved MRD-negative remission after induction and sustained molecular remission.

Conclusion: Integrating structural prediction with functional profiling enables rational therapy selection in TTMV::RARA APL.

Abstract 175

Favorable Does Not Always Mean Low Risk: Hidden Early Resistance in ETV6–RUNX1 Pediatric B-ALL

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Purpose: ETV6–RUNX1 B-ALL is considered low risk, yet some patients show early MRD persistence, suggesting concealed biological heterogeneity.

Methods: ETV6–RUNX1 B-ALL is considered low risk, yet some patients show early MRD persistence, suggesting concealed biological heterogeneity.

Results: 78.3% of ETV6–RUNX1 cases carried additional mutations. NSD2 mutation correlated with D15-MRD ≥0.1% (P=0.013). High-MRD cases showed reduced CD22/CD38, increased CD34, and transcriptomic activation of PKIB and TGF-β signaling.

Conclusion: Favorable cytogenetics do not guarantee low-risk biology. NSD2 mutation and an immune-phenotypic shift identify early resistant ETV6–RUNX1 leukemia, potentially mediated by TGF-β signaling.

Abstract 176

Clinical features and treatment ouctomes of Pediatric Hodgkin Lymphoma in a tertiary instution: A retrospective study

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Purpose: Hodgkin lymphoma accounts for 5–7% of childhood cancers and ~1% of deaths, with patterns varying by geography and socioeconomic status. While survival is high in high-income countries, data are limited in LMICs. This study describes patient characteristics and analyzes OS and EFS predictors.

Methods: A retrospective review of all HL cases from January 2013 to January 2023 was conducted. Clinical, demographic, and survival data were analyzed using Kaplan–Meier methods and Cox proportional hazards regression to estimate survival and identify prognostic factors.

Results: Among 77 patients, 5-year OS was 62.5% and EFS 60.8%. Poorer outcomes were linked to delayed diagnosis, prior TB treatment, B symptoms, advanced stage, bulky/extra-nodal disease, high LDH/ESR, delays, and poor early response. Advanced stage and high ESR independently predicted OS and EFS.

Conclusion: Advanced stage and elevated ESR were the strongest independent predictors of survival, while delayed diagnosis also adversely impacted EFS. These findings underscore the importance of early recognition, timely diagnosis, and strengthened referral pathways to improve outcomes for pediatric HL in resource-limited settings.

Abstract 177

Dynamics of the Thyroid Profile in Pubertal Boys with Acute Lymphoblastic Leukemia During Treatment

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Purpose: ALL is the most common childhood malignancy and may disrupt endocrine regulation. Metabolic and inflammatory changes can cause euthyroid sick syndrome (ESS). This study assessed TRH, TSH, T3, T4, and FT4 levels in pubertal boys with ALL before treatment and after consolidation phase III.

Methods: The study enrolled 20 pubertal boys with ALL (median age, 14.55 years) treated under the ALL MB-2015 protocol (2023–2025). Serum TRH, TSH, T3, T4, and FT4 levels were assessed before chemotherapy and after consolidation phase III using RIA and ELISA, with healthy controls (n = 20) as reference.

Results: Before chemotherapy, thyroid parameters were reduced in boys: TSH by 1.7-fold, T3 by 2.7-fold, T4 by 2.0-fold, and FT4 by 1.4-fold ($p < 0.05$); the T3/T4 ratio was also decreased, while TRH remained normal. After consolidation phase III, all parameters normalized.

Conclusion: In pubertal boys with ALL, hormonal alterations are present before therapy, suggesting possible euthyroid sick syndrome. After consolidation phase III, hypothalamic–pituitary–thyroid axis hormones normalize, indicating a functional and reversible process.

Abstract 178

ESSENTIAL THROMBOCYTHEMIA AND PREGNANCY. CLINICAL CASE

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Purpose: ET is a clonal myeloproliferative disorder. When the disease affects women during pregnancy, an adverse pregnancy outcome is possible: abortion, intrauterine fetal death, abruptio placentae, intrauterine growth retardation, and premature delivery.

Methods: This clinical case describe the management of use low molecular weight heparins in pregnancy with Essential thrombocythemia (ET).

Results: Patient had an unassisted vaginal delivery of a healthy male baby weighing 3.15 kg with an Apgar score of 8 in 1 minute and 9 in 5 minutes. The total blood loss at delivery was 200ml.

Conclusion: The pathophysiology of miscarriage in ET remains incompletely understood, but it is known that pregnancy itself is a pro-thrombotic state and that the hemostatic abnormalities related to ET may cause placental infarction and insufficiency in some cases. History of thrombosis or hemorrhage and extreme thrombocytosis in pregnant ET patients are considered to be potential risk factors for fetal and/or maternal complications. Nevertheless, there are no currently validated predictors of pregnancy outcome; for example age at pregnancy, parity, general risk factors for abortion, thrombophilia, platelet count, leukocyte count, and hemoglobin level have not been found to have significant prognostic value either in other reports. The potential capability of treatments in overcoming the effects of unfavorable prognostic factors in pregnant ET patients merits further investigation.

Abstract 179

Changes in pituitary hormonal function in pubertal boys with acute lymphoblastic leukemia during chemotherapy.

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Purpose: About 50% of childhood cancer survivors develop endocrine disorders. Data on chemotherapy effects on pituitary function in pubertal boys with ALL are limited. We assessed GH, TSH, ACTH, LH, FSH and prolactin levels before therapy and after consolidation №3.

Methods: 20 pubertal boys with ALL (median age 14.6 years) treated under ALL MB-2015 in 2023–2025 were studied. Pituitary hormones were measured before chemotherapy and after consolidation №3 by RIA/ELISA and compared with healthy controls (n=20). Analysis used Statistica 10.

Results: Before therapy, LH fell 1.5-fold; prolactin and GH rose 1.8- and 3-fold; ACTH and TSH fell 1.7-fold ($p<0.05$). After consolidation No. 3, LH normalized; FSH increased; prolactin remained high; GH dropped below baseline and normal; ACTH and TSH returned to normal.

Conclusion: ALL in pubertal boys disrupts adenohypophyseal function and trophic hormone balance. Chemotherapy does not fully normalize levels. Regular monitoring of LH, FSH, prolactin, GH, ACTH and TSH is recommended for early detection and correction of endocrine disorders.

Abstract 180

Alterations in pituitary hormone function in pubertal girls with acute lymphoblastic leukemia.

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Purpose: 50% of childhood cancer survivors develop endocrinopathies. The impact of chemotherapy (CT) on pituitary function in pubertal girls with acute lymphoblastic leukemia (ALL) is insufficiently studied. We assessed GH, TSH, ACTH, LH, FSH, and prolactin before treatment and after consolidation No. 3.

Methods: The study included 20 pubertal girls with ALL (median age 14.55 years, 11–17). Patients were treated per ALL MB-2015 (2023–2025). LH, FSH, prolactin, GH, ACTH, and TSH were measured before CT and after consolidation

No. 3. Twenty healthy pubertal girls served as controls.

Results: In girls with ALL before treatment, prolactin was increased 2.9-fold, while GH and ACTH were reduced. After consolidation No. 3, LH and FSH exceeded normal and baseline values, prolactin remained elevated, GH stayed below normal despite partial recovery, ACTH normalized, and TSH remained normal.

Conclusion: ALL in pubertal girls is associated with adenohypophyseal dysfunction and imbalance of tropic hormones. Antitumor therapy does not fully restore pituitary hormone levels. Regular monitoring of LH, FSH, prolactin, GH, ACTH, and TSH is recommended for early detection and management of endocrine disorders.

Abstract 181

Diagnostic and Prognostic Significance of the Systemic Immune-Inflammation Index in Adolescent Hodgkin Lymphoma

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Purpose: Differential diagnosis of lymphadenopathy remains challenging. HL diagnosis relies on biopsy, and response assessment mainly on PET/CT, which is not always available. The systemic immune-inflammation index (SII) may be a simple prognostic marker. This study evaluated its role in adolescents with HL.

Methods: 160 adolescents (equal sex distribution) were studied: HL, AIM, reactive LAP (n=40 each), and healthy controls (n=40). CBC with SII calculation was performed before treatment; in HL, SII was measured after each CT course. Control values were used as reference.

Results: Mean SII was 403.9 in boys and 429.5 in girls. In HL, SII increased 6.5–6.7-fold; in AIM it decreased 4.0–4.5-fold, while in reactive LAP it was normal. In HL, SII markedly decreased after the first CT course and remained low, but rose above normal in refractory or relapsed disease.

Conclusion: The SII may serve as a simple and accessible auxiliary marker for the differential diagnosis of lymphadenopathy of various etiologies in adolescents, as well as for assessing the effectiveness of antitumor therapy in Hodgkin lymphoma.

Abstract 182

Diagnostic and Prognostic Value of the Leukocyte Ratio Index in Adolescents with Hodgkin Lymphoma

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Purpose: Hodgkin lymphoma diagnosis relies on histology and immunohistochemistry, while treatment response is mainly assessed by ^18F-FDG PET/CT. Therefore, simple laboratory markers are needed. This study evaluated the clinical utility of the neutrophil-to-lymphocyte ratio (NLR) in adolescents with HL.

Methods: The study included 160 adolescents (equal sex distribution) divided into four groups (n=40 each): HL, AIM, RLA, and controls. CBC with NLR calculation was performed before treatment; in HL patients NLR was assessed after each chemotherapy course. Control values were used as reference.

Results: Mean NLR was 1.49 ± 0.08 in boys and 1.59 ± 0.13 in girls. In HL, NLR increased 3.5–4.0-fold; in AIM it decreased 2.9–3.2-fold, while in RLA it remained normal. After chemotherapy, NLR declined markedly but rose again in relapse or refractory disease.

Conclusion: NLR may serve as a simple and accessible adjunct marker for differential diagnosis of lymphadenopathies and for monitoring antitumor therapy effectiveness in adolescents with Hodgkin lymphoma.

Abstract 183

SCLEROSING CHOLANGITIS: A CHALLENGE OF LATE DIAGNOSIS OF LANGERHANS CELL HISTIOCYTOSIS IN CHILDREN IN DEVELOPING COUNTRIES — REPORT OF 3 CASES

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Purpose: Sclerosing cholangitis (SC) is a rare but severe complication of Langerhans cell histiocytosis (LCH) that can lead to progressive liver failure and the need for liver transplantation. We describe 3 cases of LCH complicated by SC, highlighting diagnostic challenges and outcomes in Uzbekistan.

Methods: We retrospectively analyzed clinical cases of LCH with liver involvement and subsequent development of SC.

Results: We report three pediatric cases in Uzbekistan with delayed diagnosis and limited access to advanced therapy. All patients had poor outcomes, highlighting the need for earlier detection and improved treatment access.

Conclusion: All 3 cases demonstrate the aggressive course of BRAF-negative LCH complicated by SC. Delayed diagnosis and limited access to transplantation and targeted therapy contributed to fatal outcomes. Early detection and improved access to advanced treatment are essential to improve prognosis in developing countries.

Abstract 184

Association of TNF-α Gene rs1800629 Polymorphism with the Risk of Primary Immune Thrombocytopenia in Children

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Purpose: To evaluate the contribution of the TNF-α gene rs1800629 polymorphism to the risk of developing primary immune thrombocytopenia in children.

Methods: Study included 89 children with ITP treated in 2017–2021 (mean age 8.7±1.7 years). TNF-α rs1800629 genotyping by PCR with restriction analysis (Applied Biosystems 2720). Statistics: OpenEpi 9.3, χ^2, OR, 95% CI; $p<0.05$.

Results: Allele distribution of rs1800629 differed in ITP. The G allele was less frequent (OR=0.41; p=0.012), while the A allele was more frequent (OR=2.43; p=0.012). The G/A genotype was increased in patients (OR=2.55; p=0.014). A/A was higher but not significant (p=0.29).

Conclusion: The TNF-α gene rs1800629 polymorphism is associated with an increased risk of primary immune thrombocytopenia in children. Carriage of the A allele and G/A genotype may be considered genetic susceptibility factors for the disease.

Abstract 185

Early Differentiation of Fungal from Bacterial Infections in Children With Hematologic Malignancy: A Single center case-control study

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Purpose: Traditional diagnostic approaches may delay the identification of invasive fungal infections (IFI) in children with hematological malignancies (HM) due to inherent limitations. We evaluated the effectiveness of clinical indicators in differentiating IFI from bacteremia in this pediatric population.

Methods: A case group of 50 patients with acute leukemia or lymphoma who developed probable or proven IFI following chemotherapy and had negative bacterial cultures was selected. A control group of 97 patients who developed bacteremia without IFI post-chemotherapy was also included.

Results: Prolonged antibiotic use (≥7 days), time since chemotherapy, and fever duration were independent predictors of IFI. A model incorporating these factors achieved an AUC of 0.938, with 85.1% sensitivity and 87.5% specificity.

Conclusion: The combination of prolonged antibiotic use ≥ 7 days, time since chemotherapy, and fever duration before diagnosis effectively differentiated IFI from bacteremia in pediatric patients with HM.

Abstract 186

Course of refractory clostridioides difficile colitis with subsequent transformation into resistant acute gastrointestinal graft-versus-host disease (GI-aGVHD) after a second haploidentical hematopoietic stem cell transplantation

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Purpose: The aim of this report is to present a clini cal case of a patient with myelodysplastic syndrome who underwent two consecutive haploidentical hematopoi etic stem cell transplantations (HSCT), complicated by refractory Clostridioides difficile colitis and subsequent transformation into resistant acute gastrointestinal graft versus-host disease (GI-aGVHD). Additional objectives included evaluating the effectiveness of desensitization strategies, anti infective therapy, biological agents, and analyzing factors contributing to immune graft rejection after the first HSCT and the complex immuno-infectious course following the second transplantation.

Methods: The patient with myelodysplastic syndrome underwent an initial haploidentical HSCT from his bro ther following conditioning with fludarabine and busul fan (8 mg/kg) combined with desensitization (rituximab, mycophenolate mofetil, two sessions of plasmapheresis, IVIG). After immune graft rejection, a second haploiden tical HSCT was performed using his father as donor with a modified desensitization regimen (daratumumab, plas mapheresis, IVIG, cyclosporine A) and conditioning with fludarabine and melphalan (100 mg/m2). Antibacterial therapy was administered via stepwise escalation including meropenem, amikacin, linezolid, tigecycline, and piperacillin/tazobactam; antifungal pro phylaxis was adjusted from caspofungin to voriconazole. Cytomegalovirus (CMV) monitoring was conducted via PCR with corresponding antiviral therapy (ganciclovir, foscarnet, valganciclovir). C. difficile infection was diag nosed via toxin A/B detection. Treatment included van comycin, metronidazole, rifaximin, tigecycline, and fidaxomicin, as well as two fecal microbiota transplantations (FMT). Upon suspicion of acute GVHD, therapy included corticosteroids, ruxolitinib, infliximab, vedolizumab, and ustekinumab.

Results: The first haploidentical HSCT was compli cated by early febrile neutropenia requiring multiple antimicrobial escalations. Persistent pancytopenia and presence of anti-HLA antibodies led to confirmed immu nologic graft rejection. Following the second HSCT, the patient experienced recurrent febrile episodes during profound agranulo cytosis, requiring combined antibacterial therapy and central venous catheter replacement. By Day +24, the first CMV reactivation (5,600 copies/mL) was detected, followed by two further reactivations, one with ganciclo vir resistance, requiring switching to foscarnet and later valganciclovir. On Day +26, the patient developed liquid stools up to 11 times per day with confirmed C. difficile toxins A/B. Multistep therapy including vancomycin + metronida zole, two FMT procedures, a combined regimen, and fi daxomicin 200 mg twice daily led to toxin eradication only by Day +100. On Day +28, progressive diarrhea and increased stool volume raised suspicion of acute GIaGVHD. Despite treatment with methylprednisolone and ruxolitinib, the disease advanced to grade IV. Infliximab administration failed to achieve sustained response. Under profound im munosuppression, clinical deterioration continued, with stool frequency increasing to 25 per day. Two infusions of vedolizumab were administered, followed by ustekinu mab as salvage therapy.

Conclusion: This case illustrates a complex multifac torial post transplant course after two haploidentical HSCTs in a patient with smyelodysplastic syndrome, complicated by refractory C. difficile colitis, recurrent infections, and resistant GI-aGVHD. The C. difficile in fection demonstrated marked resistance to standard therapy and FMT, requiring a multistep combined treatment strategy including fidaxomicin. Acute gas trointestinal GVHD exhibited profound resistance to all therapeutic lines, necessitating the use of biological agents (infliximab, vedolizumab, ustekinumab). This case underscores the importance of early detection of overlapping infectious and immunologic complications and highlights the need for individualized, multidisci plinary management strategies in heavily immunocom promised patients undergoing repeated haploidentical HSCT.

Abstract 187

Clinical and Laboratory Aspects of Hematopoietic Stem Cell Mobilization and Apheresis Collection in Pediatric Patients with Malignancies

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Purpose: Autologous hematopoietic stem cell transplantation (auto-HSCT) plays a critical role in the multimodality treatment of malignant and hematological diseases in pediatric patients. Auto-HSCT ensures hematopoietic recovery following high-dose chemotherapy and facilitates remission consolidation. A key stage of transplant preparation is the mobilization and harvesting of hematopoietic stem cells (HSCs). In pediatric practice, these processes may be challenged by technical and physiological constraints associated with age-related patient characteristics. To evaluate the efficacy of HSC mobilization and apheresis collection in children with malignancies based on an analysis of clinical and laboratory parameters.

Methods: The study was conducted at a specialized department of transfusiology, HSC processing, and storage. Six HSC apheresis procedures were analyzed in oncology patients (neuroblastoma, n=5; lymphoma, n=1). Hematopoietic mobilization was performed followed by cell collection using apheresis. Prior to the procedures, comprehensive clinical, laboratory, and instrumental examinations were conducted. Laboratory monitoring included a complete blood count (CBC) to assess hemoglobin concentration, leukocyte and platelet counts, and the dynamics of hematopoietic recovery post-polychemotherapy. The CD34+ cell count in peripheral blood was determined by flow cytometry starting from the third day of stimulation, with subsequent dynamic monitoring until threshold values for apheresis initiation were reached. To select the optimal vascular access, vascular ultrasound was performed. All interventions were conducted after obtaining informed voluntary consent from the patients' legal representatives. Apheresis was performed using the Spectra Optia cell separator (Terumo BCT Inc., USA) utilizing continuous and intermittent separation modes. The use of extracorporeal circuit priming with red blood cell units ensured hemodynamic stability in low-weight patients.

Results: Successful mobilization of hematopoietic stem cells was recorded in 5/6 patients (83.3%) within 1–4 days after 3–5 cycles of polychemotherapy; in 1/6 patients (16.7%), it occurred on the 5th day post-surgery. Apheresis initiation: days 2–4 in 4/5 cases (80.0%); day 5 in 1/5 cases (20.0%); no cases of initiation on day ≥6 were recorded. The median time to apheresis initiation was 3 days [IQR: 2–4]. CD34+ cell yield ($\times 10^6$/kg): <2 in 1/6 (16.7%); 2–3.9 in 2/6 (33.3%); 4–10 in 2/6 (33.3%); >10 in 1/6 (16.7%). The median CD34+ cell yield was ~4.5×10^6/kg [IQR: 2.5–8.0]. The target graft cellularity (≥2×10^6/kg) was achieved in 5/6 patients (83.3%).

Conclusion: Mobilization and apheresis collection of hematopoietic stem cells in children with malignancies demonstrate high efficacy: the target CD34+ cell level (≥2×10^6/kg) was achieved in 83.3% of patients. In most cases, apheresis was initiated early (median — 3 days [IQR: 2–4]), indicating predictable mobilization kinetics under standard stimulation regimens. The results confirm that the use of modern apheresis technologies, combined with dynamic clinical and laboratory monitoring, ensures adequate cellular yield and an acceptable safety profile in pediatric patients.

Abstract 188

Adalimumab in the treatment of intestinal acute graft-versus-host disease in children

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Purpose: Hematopoietic stem cell transplantation (HSCT) as a therapy is constantly evolving, primarily through the expansion of therapeutic arsenal, including the introduction of innovative immunosuppressive regimens extrapolated from other fields of medicine, particularly gastroenterology,

immunology, and rheumatology. Despite significant advances in the prevention and treatment of acute graft-versus-host disease (aGVHD) in recent decades, severe forms of this disease remain a major cause of morbidity and mortality after HSCT. Our study describes the results of treating the intestinal form of acute GVHD with the TNF-α inhibitor adalimumab, a human IgG1 monoclonal antibody. This drug is included in clinical guidelines for the treatment of ulcerative colitis and Crohn's disease in children and adults. Experience with the use of adalimumab in HSCT is extremely limited. Aims: To demonstrate experience with adalimumab in children with intestinal aGVHD.

Methods: This is the first Russian study to present results with the use of adalimumab in children with intestinal aGVHD. From July 2025 to March 2026, 5 children (including 4 boys) received adalimumab: 3 patients with acute lymphoblastic leukemia, 1 patient with acute myeloid leukemia, and 1 patient with primary immunodeficiency. The median age at the time of HSCT was 10 (range 4–16) years. Haploidentical HSCT was performed in 3 children, in the remaining cases - unrelated HLA-identical. Native peripheral blood stem cells were used as a graft in 4 patients, bone marrow - in 1. Cellular characteristics of the grafts: CD34+ 14.9 (3.46–19.28) × 10^6 /kg, CD3+ 5.0 (0.83–5.3) × 10^8 per kg of recipient body weight. GVHD prophylaxis/therapy included: Janus kinase inhibitors, cyclophosphamide, abatacept, rituximab, vedolizumab, tocilizumab, etanercept. The indication for the introduction of adalimumab as a third-line drug was the presence of clinical manifestations of the intestinal form of aGVHD, according to the MAGIC criteria. The dosing regimen was selected according to clinical guidelines for the treatment of ulcerative colitis in children.

Results: Leukopoiesis was restored in all patients within 15 (12-20) days, and complete donor chimerism persisted from day 30 to the present. Three of the five children demonstrated a clinical response after the first adalimumab administration. One child demonstrated a clinical response after the second administration, and despite gastrointestinal tract contamination with Ps. aeruginosa and adenoviremia, escalation of immunosuppression did not affect resolution of the infection. The fifth patient, with grade 3-4 hepatic and intestinal aGVHD, achieved grade 1 after the seventh adalimumab administration. All children are currently alive, and four do not require hospitalization.

Conclusion: Adalimumab can be considered in the arsenal of drugs for the treatment of aGVHD, but its use requires careful monitoring of infectious risks when integrated into the structure of combination immunosuppressive therapy.

Abstract 189

ANNUAL RETROSPECTIVE ANALYSIS OF CLINICAL AND DIAGNOSTIC FEATURES OF PEDIATRIC LYMPHOMA: A SINGLE-CENTER EXPERIENCE IN UZBEKISTAN

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Purpose: Pediatric lymphomas present a significant diagnostic challenge due to their heterogeneous clinical manifestations and frequent delays in initial detection. Comprehensive evaluation of clinical features, including extranodal involvement, is critical for improving early diagnosis and optimizing therapeutic strategies. Objective: To evaluate the clinical and diagnostic characteristics of pediatric lymphomas based on a retrospective analysis of patients treated during 2025.

Methods: A retrospective study was conducted on 40 pediatric patients diagnosed with lymphoma in 2025. The analysis included demographic data, histological subtypes, disease staging (including the presence of B-symptoms), lymph node distribution, and extranodal involvement. Diagnosis was confirmed via clinical, laboratory, and advanced imaging modalities, with morphological verification performed through immunohistochemistry (IHC).

Results: The cohort consisted of 26 males (65%) and 14 females (35%). Hodgkin lymphoma (HL) was identified in 22 patients (55%), while non-Hodgkin lymphoma (NHL) accounted for 18 cases (45%). Systemic B-symptoms were present in 30% (n=12) of the patients. The most frequent sites of lymphadenopathy were: Cervical: 92.5%, Mediastinal: 87.5%, Axillary: 80%, Abdominal: 75%. Extranodal disease was identified in 65% (n=26) of cases. The most common extranodal sites included the bone marrow (65%), spleen (32.5%), and liver (22.5%). In several instances, involvement of multiple extranodal sites indicated advanced-stage disease.

Conclusion: Pediatric lymphomas in this cohort demonstrated diverse clinical presentations with a high prevalence of multi-regional lymph node involvement, particularly in the cervical and mediastinal areas. The high rate of extranodal involvement, specifically bone marrow infiltration, underscores the frequency of advanced-stage presentation. These findings highlight the urgent need for early diagnosis through molecular-genetic verification and the timely initiation of risk-stratified therapy.

Abstract 190

IMMUNOHISTOCHEMICAL CHARACTERISTICS OF PEDIATRIC BURKITT LYMPHOMA: A SINGLE-CENTER EXPERIENCE IN UZBEKISTAN

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Purpose: Pediatric Burkitt lymphoma is a highly aggressive neoplasm characterized by a rapid growth rate, necessitating urgent and precise diagnosis. Immunohistochemical (IHC) analysis is fundamental for diagnostic verification and for differentiating Burkitt lymphoma from other B-cell lymphomas. Objective: To evaluate the immunohistochemical features of pediatric Burkitt lymphoma based on an analysis of 12 clinical cases.

Methods: A retrospective analysis of 12 medical records of pediatric patients diagnosed with Burkitt lymphoma was conducted. Disease staging was determined using the St. Jude (Murphy) classification. The study analyzed patient demographics (age and sex), clinical stage, and IHC markers, including MYC, BCL2, BCL6, and Ki-67. Diagnosis was established through morphological and IHC examination.

Results: The study included 12 patients, with a male predominance (n=8, 67%) compared to females (n=4, 33%). Most cases were diagnosed at advanced stages (III–IV). IHC analysis revealed MYC expression in 11 patients (92%), while BCL2 expression was absent in all cases (100%). BCL6 expression was identified in 11 patients (92%). A high level of proliferative activity (Ki-67 ≈ 100%) was observed in all patients. In one case, MYC expression was focal, and in another, it was absent. Although MYC and BCL6 expression were present, the diagnosis of "double-hit" lymphoma could not be confirmed without molecular-genetic studies. TP53 expression and EBV status were not assessed in this study.

Conclusion: The IHC profile of pediatric Burkitt lymphoma in this cohort is characterized by high MYC expression, absence of BCL2, and a high Ki-67 proliferation index, aligning with classical diagnostic criteria. To optimize risk stratification and outcome prediction, the implementation of mandatory FISH testing for all newly diagnosed patients is planned to identify "double-hit" and "triple-hit" lymphomas.

Abstract 191

Survival of Pediatric Acute Lymphoblastic Leukemia in Uzbekistan: A Review of Available Evidence

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Purpose: Acute lymphoblastic leukemia (ALL) is the most common childhood malignancy, with survival exceeding 85% in high-income countries. However, outcomes in Uzbekistan remain underreported and are likely lower due to healthcare system limitations. This study aimed to summarize available evidence on survival outcomes and identify key prognostic factors affecting pediatric ALL patients.

Methods: A narrative literature review was conducted using published studies on pediatric acute lymphoblastic leukemia in Uzbekistan and comparable low- and middle-income countries. Relevant articles were identified through electronic databases and regional reports. Data on survival outcomes, including overall survival and event-free survival, as well as prognostic factors, were extracted and qualitatively analyzed.

Results: Available evidence from Uzbekistan suggests a 3-year event-free survival (EFS) of approximately 64.5% in pediatric acute lymphoblastic leukemia patients, which is lower than survival rates reported in high-income countries

(>85–90%). Poor prognostic factors include high leukocyte count at diagnosis, poor early treatment response, and advanced disease features. Additional contributors to reduced survival include treatment-related infections, delayed diagnosis, and limited access to specialized care.

Conclusion: Survival outcomes of pediatric acute lymphoblastic leukemia in Uzbekistan remain below global benchmarks. Strengthening early diagnosis, improving supportive care, and expanding access to risk-adapted therapy are essential to improve outcomes and reduce treatment-related mortality.

Abstract 192

The outcomes of neutropenic fever in Acute Lymphoblastic Leukemia patients in the induction phase

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Purpose: Less than 60% of the children worldwide have access to cancer treatment, and the outcomes for Acute Lymphoblastic Leukemia (ALL) in low-income countries remain poor. Patients with acute leukemia are highly susceptible to infectious diseases especially in the induction phase with the intense chemotherapy. Patients with chemotherapy-induced neutropenia are at particularly high risk, and microbiological agents include viral, bacterial, and fungal agents.

Methods: This is a retrospective study. All children diagnosed with ALL and attending the pediatric oncology ward at Angkor Hospital for Children from August 2018 to September 2021 were included. Information regarding demographics, laboratory, microbiological profile and outcomes were extracted and reviewed.

Results: Of the total 50 ALL patients, 27 cases had neutropnic fever in the induction phase of the chemotherapy. Among 27 patients, 16 were males and 11 were female. B-cell leukemia (pre-B-ALL) was diagnosed in 92.5% (n=25), while 7.4% (n=2) had acute T-cell leukemia (pre-T-ALL). Patients were classified in high risk 55.5% (n=15) and low risk 44.5% (n=12). The major sites of infection were lung 14.8% (n=4) and urinary tract 14.8% (n=4), there was neither clinical nor laboratory evidence of infection fever of unknown origin 51.8% (n=14). Blood cultures were positive in 8 patients (29.6%) with 14 organisms grew. The most common organisms isolated were candidas tropicalis 21.4% (n=3), candidas albican 21.4% (n=3) followed by Enterobacter spp 14.2% (n=2). Twenty five (92.5%) patients resolved without sequelae. Overall induction mortality due to neutropenic fever was 7.4% (n=2), manly by fungal infection

Conclusion: Infections are the major cause of mortality and morbidity in patients with ALL on induction chemotherapy. Based on our findings, the outcomes are good for the majority of patients if we detect earlier with the prompt antimicrobial agents.

Abstract 193

Belumosudil in pediatric refractory chronic Graft-versus-Host Disease after Allogeneic Hematopoietic Stem Cell Transplantation: single center experience in 12 children

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Purpose: Chronic graft versus host disease (cGvHD) is among the most common, severe, and life threatening complications following allogeneic hematopoietic stem cell transplantation (allo HSCT). The management of steroid refractory (SR) cGvHD in children remains challenging and necessitates novel therapeutic approaches. Belumosudil is a selective inhibitor of Rho associated coiled coil containing protein kinase 2 (ROCK2), developed specifically for the treatment of cGvHD. Inhibition of the ROCK2 signaling pathway by belumosudil may represent an effective and safe strategy for managing refractory cGvHD in the pediatric population.

Methods: We report data on 12 patients with refractory cGvHD treated with belumosudil since 2023. All patients underwent allo HSCT at the N.N. Blokhin National Medical Research Center. The median age was 12 years (range, 2 to 18 years), with a male to female ratio of 10:2. Underlying primary diseases included acute lymphoblastic leukemia (59%, n=7), acute myeloid leukemia (25%, n=3), myelodysplastic syndrome (8%, n=1), and Fanconi anemia (8%, n=1). Donor types were haploidentical (33%, n=4) and matched unrelated donor (67%, n=8). Patients undergoing TCRαβ/CD19 depleted grafts did not receive standard immunosuppressive therapy (IST). For GvHD prophylaxis with post transplant cyclophosphamide, IST included cyclosporine A or ruxolitinib. In matched unrelated donor recipients, GvHD prophylaxis consisted of anti thymocyte globulin in combination with tacrolimus or ruxolitinib. All patients treated with belumosudil had a history of more than two prior lines of IST. Overlap syndrome was present in 41% (n=5) of patients. The most commonly involved organs were skin (75%, n=9), mucous membranes (66%, n=8), lungs (58%, n=7), eyes (41%, n=5), liver (33%, n=4), gastrointestinal tract (25%, n=3), muscles and fascia (16%, n=2), and joints (16%, n=2). Doses ranged from 50 mg to 200 mg per day, depending on patient weight and clinical status.

Results: At the 1 month assessment, a complete response was achieved in 25% (n=3) of patients, a partial response in 50% (n=6), and no response in 25% (n=3); the latter were transitioned to alternative therapy. By the 3 month follow up, no patient had achieved a complete response. A partial response was maintained in 50% (n=6) of patients, while 16% (n=2) showed no response. The most common adverse events were infectious complications, predominantly pneumonia. One patient developed uncontrolled emetic syndrome.

Conclusion: Preliminary data suggest that belumosudil may be a promising therapeutic option for refractory cGvHD in children, particularly in cases with fibrosing manifestations. Our single center experience demonstrates that inhibition of the ROCK2 pathway represents a novel pathogenetic approach in pediatric patients. Larger scale prospective studies are warranted to confirm efficacy and to establish the optimal dosage and duration of belumosudil therapy in the pediatric population.

Abstract 194

Genetic Landscape of pB-Lymphoblastic Lymphoma in Children and Adolescents

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Purpose: Lymphoblastic lymphoma (LBL) stands as the second most prevalent NHL subtype among children. Precursor B cell LBL (pB LBL) constitutes up to 25% of all LBL cases. According to WHO classification, pB LBL and B ALL share morphological, immunophenotypic, and genetic features but unlike in B ALL, the prognostic implications of genetic factors within pB LBL remain undefined.

Methods: The analysis encompassed 131 patients diagnosed with pB LBL between 2022 and 2025. The cohort comprised an equal representation of males and females, with a median age of 8.3 years (0.4 - 17.1). Copy number variations (CNVs) were examined in 58 patients through MLPA (SALSA P335 ALL-IKZF1 and P202-C2 IKZF1-ERG kits). Genome-wide structural variants were investigated using a modified Hi-C sequencing approach in 20 patients. Risk stratification was determined based on genetic rearrangements (Moorman's criteria (2014)).

Results: The median follow up period extended to 2.1 years (0–21.6). The OS rate was 89.6%, EFS - 71.4%. Female sex emerged as a significant predictor of better EFS (p=0.029; HR 0.39; 95% CI 0.16–0.94). Disease stage, bone marrow, CNS involvement, patient age did not demonstrate an impact on either EFS or OS. Among the genetic abnormalities identified were deletions in CDKN2A/2B (36.2%), RB1 (24%), and PAX5 (22.4%). A comparative analysis with B-ALL revealed a higher incidence of RB1 (p<0.0001) and IKZF1 (p=0.0058) deletions in pB-LBL. Hyperdiploidy, IKZF1 deletions, the Moorman risk groups did not iinfluence outcomes. In relapsed patients (n=4), high-risk B-ALL rearrangements (KMT2A (KMT2Ar), BCR::ABL1, MYC::IGH), were identified, three of these patients relapsed, two of them died.

Conclusion: Despite morphological resemblance to B-ALL, pediatric pB-LBL warrants recognition as a distinct entity with unique molecular profile. Comprehensive genetic analysis can illuminate the presence of high-risk rearrangements, but the current utility of a CNV-based genetic classifier in this population is not yet justified, highlighting the need for further research.

Abstract 195

Ravulizumab for refractory transplant-associated thrombotic microangiopathy in pediatric patients: a single-centre experience

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Purpose: Diagnosis and management of transplant-associated thrombotic microangiopathy (TA-TMA) in pediatric patients remain challenging, especially in cases refractory to first-line complement inhibition with eculizumab. Ravulizumab (a C5 complement inhibitor), offers a potential therapeutic advantage in such settings. Real-world data on ravulizumab usage for the pediatric population are currently limited. We aimed to report our single-center experience with ravulizumab for the treatment of severe refractory TA-TMA in pediatric patients following allogeneic hematopoietic stem cell transplantation (allo-HSCT).

Methods: Among 347 pediatric patients who underwent HSCT at the Nikolay Blokhin National Medical Research Center of Oncology between 2023 and 2024, seven were diagnosed with TA-TMA according to EBMT criteria. Of these, three patients with severe refractory TA-TMA — defined as absence of clinical and laboratory response after a median of 4 eculizumab infusions — were subsequently treated with ravulizumab at the weight-adjusted recommended dose.

Results: All three patients had leukemia (AML, n=2; ALL, n=1) and underwent allo-HSCT from a matched unrelated donor (MUD). Female predominance was noted (M:F = 1:2); ages were 17, 17, and 12 years. Conditioning regimens included TBI/VP/Flu (ALL patient) and Treo/Flu/Mel (AML patients). GvHD prophylaxis comprised ATG, rituximab, abatacept, and tacrolimus or ruxolitinib in all cases. TA-TMA onset varied: day +77 in patient 1 (accompanied by renal bleeding), and days +280 and +273 in patients 2 and 3, respectively, both in the context of severe chronic GvHD. Following switch to ravulizumab (median 4 infusions), all three patients demonstrated clinical and laboratory response. No severe infusion-related adverse events were observed.

Conclusion: Ravulizumab represents a promising rescue therapy for pediatric patients with refractory TA-TMA after allo-HSCT. Our single-center experience supports its feasibility and tolerability in this population. Larger prospective multicenter studies are needed to further define the role of ravulizumab in pediatric TA-TMA management and to validate its long-term outcomes.

Abstract 196

Suboptimal infrastructure & economic constraints: deterrents to outcomes in relapsed Pediatric Acute Lymphoblastic Leukemia

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Purpose: Relapse remains the leading cause of treatment failure in Acute Lymphoblastic Leukemia (ALL). Survival in ALL has plateaued, the current challenge lying in reducing relapse rates. Management of relapse remains particularly arduous in children from a poor socioeconomic background and within a resource-limited setting. Analysis of the pattern and outcome of relapsed ALL at our centre is presented.

Methods: Children <13 years treated for ALL between December 2017 and December 2021 who relapsed were analysed. Pattern, site of relapse and outcomes were analyzed.

Results: A total of 566 patients were treated over 4 years, of whom 139 (24.5%) relapsed. Very early relapse (VER) occurred in 42 (30.2%), early relapse (ER) in 56 (40.3%), and late relapse (LR) in 41 (29.5%). Isolated medullary relapse was seen in 66 (47.5%), extramedullary in 54 (38.8%), and combined relapse in 19 (13.7%). Ninety patients (65%) opted for palliative care, while 49 (35%) received curative treatment (VER 14.3%, ER 32%, LR 61%). Of these, 37 received chemotherapy and 12 underwent HSCT. Distribution was: VER (CT 4, HSCT 2), ER (CT 11, HSCT 7), and LR (CT 22, HSCT 3). There were 18 deaths (13 treatment-related, 5 disease progression). Six (12.2%) relapsed, 3 (6.1%) abandoned therapy, and 22 (45%) remain well. Two-year EFS was 45% overall, higher with HSCT (75%), and by subgroup: LR 48%, ER 44%, VER 33%. Among HSCT recipients, 75% survived.

Conclusion: Outcomes of relapsed ALL in LMICs are poor. A large percentage of patients decline curative treatment, primarily due to guarded prognosis, financial constraints, and limited access to HSCT services. Although government funding for cancer care has increased, it is still not all encompassing. Comprehensive strategies, including funding for HSCT, expansion of public sector pediatric oncology services are required to ensure equity in care and improve outcomes.

Abstract 197

Pediatric Acute Myeloid Leukemia: From High Mortality to Better Survival

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Purpose: Purpose Treatment-related mortality (TRM) is a major impediment to survival in LMIC's being a modifiable factor toward improving outcomes .This analysis evaluated the outcome of pediatric non-M3 AML over the last 20 years.

Methods: Retrospective review of 100 patients between 2004-2013 (Group A[A]) and 47 treated from 2021-2024 (Group B[B]). Abandonment was 35% in A, 14% in B . Outcomes were compared.

Results: Mean age: A: 7.3± 3.3; B: 4.7 ± 3.3 years; male to female ratio being similar (3:2). Cytogenetic abnormalities identified in B in 39 patients : t(8;21) in 14 (25%), t(8;21)+FLT3 in 2 , MLDS in 6 , inv(16) in 3, FLT3-ITD, TKD, monosomy 7 , and RBM15 in 1 each. 16 had no abnormality. CNS involvement in 8% and 7% in A & B. 64% achieved remission after course 1 , with five having refractory disease in group A and 65% achieved MRD negativity, 13 (32%) were MRD positive, after course 1 in B Group A: 25 died during course 1, with 8, 7 & 8 deaths after course 2, 3 & 4. Sepsis accounted for 98% , TRM being 48%. Group B : 9(19%) deaths , 6 after course 1, 2 after course 2 and 1 after consolidation. Sepsis attributed to 77% death. Relapse rate was similar in both groups 20% and 21%; median time: 10 months. Survival: Event free survival was 25.3% and 47% at 3.5 years in A and B .

Conclusion: An improved outcome from 25.3% to 47% over a decade is attributed to increase in supportive care services and dedicated personnel. Increase in funding by government and NGO help has helped to decrease abandonment rates.TRM has diminished, but is still unacceptably high. Combating infections in low- and middle-income countries remains a critical challenge, emphasizing the need for robust infection-control strategies.

Abstract 198

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR SECONDARY ACUTE LEUKEMIA: SINGLE CENTER EXPERIENCE FOR 8 CHILDREN

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Purpose: Treatment of secondary acute leukemias (AL) following prior solid malignancies remains a significant challenge. Allogeneic hematopoietic stem cell transplantation (aHSCT) is an essential component of therapy for secondary AL but is associated with toxicity, a high risk of graft-versus-host disease (GVHD), and other transplant-related complications

Methods: This retrospective study analyzes the experience of aHSCT in children with secondary AL at the N.N. Blokhin National Medical Research Center of Oncology between 2022 and 2026. The aim was to evaluate the safety, tolerability, and efficacy of aHSCT in a single-center pediatric cohort.

Results: Eight children (M:F = 5:3) with secondary AL after prior solid tumors were included. The median age at diagnosis of secondary AL was 9 (range 2–16) years. Diagnoses included acute myeloid leukemia (AML) in 7 patients and acute lymphoblastic leukemia (ALL) in 1 patient. All patients had KMT2A rearrangements and were in remission prior to HSCT. Peripheral blood stem cells were used in all cases. Donors included haploidentical with TCR α/β/CD19 depletion (n=1), haploidentical with post-transplant cyclophosphamide (n=2), matched unrelated (n=3), and matched related (n=2). Conditioning was

myeloablative (treosulfan, thiotepa, fludarabine). Defibrotide was used for toxicity prophylaxis. GVHD prophylaxis (without TCR α/β/CD19 depletion) included calcineurin inhibitors or ruxolitinib, as well as abatacept, rituximab, ATG and vedolizumab. All patients achieved engraftment (median 14 [12–18] days). GVHD occurred in 1 patient (after unrelated donor HSCT). Infectious complications were observed in 4 patients; one patient developed TA-TMA. Only 1 patient died due to transplant-related CNS toxicity. Seven patients remain alive in complete remission. Median follow-up was 14 (4–36) months.

Conclusion: aHSCT in secondary AL carries risks of post-transplant complications but remains the only curative option. Non-relapse mortality was relatively low, and acceptable outcomes with manageable toxicity were achieved in this cohort.

Abstract 199

Preliminary Evaluation of the Effectiveness of Allogeneic Stem Cell Transplantation in Treating Severe Thalassemia at Hue Central Hospital

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Purpose: Thalassemia is highly prevalent in Vietnam with 2.000 new severe cases yearly. Bone marrow transplantation remains the only established long-term cure for severe thalassemia. This report describes the baseline characteristics of severe thalassemia and evaluates the feasibility and effectiveness of bone marrow transplantation therapy at Hue Central Hospital, Vietnam.

Methods: This is a prospective, interventional cohort study conducted at Hue Central Hospital, Vietnam for severe thalassemia who received allogeneic stem cell transplantation between September 2024 (start of program) and December 2025. Data were analyzed in SPSS v.18.0

Results: A total of 14 matched-related BMTs have been performed during this time. Now, all of whom are disease- and GVHD-free. The median age at BMT was 5.9 years (2.0-10.0). The male and female accounted for 57.1% and 42.9%, respectively. There were 9 cases with HbE/Beta-Thalassemia and 5 cases with Alpha-Thalassemia. The median pre-transplant ferritin level was 1320.9 ng/mL (236-2917). The median dose of nucleated cells was 9.4x108 cells/kg (8.4-10x108). The median platelet and neutrophil engraftment time was 20.7 days (16-26) and 19.3 days (13-26), respectively. There were four cases (33.3%) with skin aGVHD-grade I and four cases (33.3%) with hemorrhagic cystitis. The median chimerism after day 30 was 96.1%. The median Hb after transplant was 12.7 (10.6-14.7) g/dl. All patients are now very healthy, without red blood cell transfusions, and no GVHD.

Conclusion: Allogeneic HSCT from HLA-matched sibling donors is a safe and curative treatment for severe thalassemia. The high GVHD-free and thalassemia-free survival improves long-term health-related quality of life in patients with severe thalassemia. Early transplantation may be highly cost-effective compared with lifelong transfusion and chelation in the Vietnamese health system.

Abstract 200

Are t(8;21) and inv(16) truly equivalent in pediatric core-binding factor acute myeloid leukemia? Experience from a Tertiary Cancer Center in India

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Purpose: t(8;21) and inv(16)-AML are conventionally classified together as core-binding factor AML (CBF-AML). However, emerging data suggest that the 2 entities are biologically and prognostically distinct. We report the differential biological features and outcomes of 2 genetic subtypes of pediatric CBF-AML patients treated at our center over a decade.

Methods: The retrospective audit captured demographics, cytogenetics, CNS status, treatment response, and survival of patients (≤18y) with CBF-AML (2012-2022). Diagnosis was based on morphology and karyotyping. Treatment consisted of anthracycline-cytarabine induction (x 2) and high-dose cytarabine consolidation (x 2). Response evaluation included marrow morphology and, from 2019 onwards, molecular MRD assay using nested PCR.

Results: Analyses include 41 patients: 30 with t(8;21) and 11 with inv(16). The median leukocyte counts (x109/L) at diagnosis were 12.9(7.15,22.4) and 131.7(67.6,326.9) for t(8;21) vs. inv(16)-AML($p<0.00001$). Six (55%) patients with inv(16) vs. none with t(8;21) had hyperleukocytosis ≥100x109/L($p<0.001$). Age ($p=0.64$), sex ($p=0.73$), and frequency of CNS 2/3 status ($p=1.00$) were comparable. Secondary cytogenetic aberrations (SCAs) were more prevalent in t(8;21) (24/30; 80%) than in inv(16) (5/11; 45.4%) ($p=0.048$). Deletions vs. trisomies were the most common SCAs in the t(8;21) vs. inv(16) group. Morphological remission was achieved in 29/30 (97%) with t(8;21) and in all with inv(16) post-course-1 induction. Limited available results of MRD suggested comparable MRD responses ($p=0.33$ and 0.2). Ten patients relapsed, 9/30 (30%) with t(8;21) vs. 1/11 (9%) with inv(16) ($p=0.25$). At a median follow-up of 4.46 years, treating non-relapse mortality as the competing event, the 4-year cumulative incidence of relapse for t(8;21) vs. inv(16)-AML was 47.3%(95%CI:23-68.7) vs. 11.2%(95%CI:0.46-42.3)(Gray's test $p=0.08$). The 4-year relapse-free survival and overall survival estimates for t(8;21) vs. inv(16)-AML were 49.6%(±12.2%) vs. 88.9(±10.5%) and 64.1%(±11.4%) vs 80.8%(±12.2%), respectively (log-rank pRFS: 0.09, pOS: 0.23).

Conclusion: Pediatric t(8;21)-AML tends to have a higher cumulative incidence of relapse than inv(16)-AML, thus questioning the convention of grouping t(8;21) and inv(16)-AML in a single basket.

Abstract 201

MEASURABLE RESIDUAL DISEASE CLEARANCE AND SHORT-TERM TOXICITY OF BLINATUMOMAB IN PEDIATRIC RELAPSED/REFRACTORY PRECURSOR B-CELL ACUTE LYMPHOBLASTIC LEUKEMIA: A SINGLE-CENTER REAL-WORLD EXPERIENCE FROM INDIA

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Purpose: Data on the efficacy and safety of blinatumomab in low-and middle-income countries are limited. We report our experience with blinatumomab, focusing on the measurable residual disease (MRD) response and short-term toxicities.

Methods: Demographics, indications for blinatumomab, MRD-response, and short-term toxicities were collected retrospectively. Patients (1-18 y) with medullary relapse or refractory MRD-positive B-cell precursor ALL received one or two 28-day cycles of blinatumomab infusion at 15 mcg/m2/d (5 mcg/m2/d, during days 1-7 of Cycle-1). All patients received anti-seizure prophylaxis. MRD was estimated by flow cytometry in all patients and by qPCR in a subset. Toxicities were graded according to the Common Terminology Criteria for Adverse Events (CTCAEv5.0).

Results: Between Nov 1, 2020, and Dec 31, 2025, 45 patients [median age: 7.8 years (IQR: 5.85,10.77)] received a median of 2 cycles of blinatumomab for relapsed (n=29) or refractory frontline disease (n=16). Pre-blinatumomab, all patients were in morphological remission; 16 (36%) were MRD-positive. The MRD-negativity was 50%(8/16) and 81%(13/16) post-Cycle-1 and -2, respectively. Of 36 patients with MRD-negativity post-Cycle-1, 3(8.3%) became MRD-positive post-Cycle-2. MRD-positivity declined from 16/45(36%) pre-blinatumomab to 6/45 (13%) after two cycles (McNemar $p=0.02$). The incidence of cytokine release syndrome (CRS), neurotoxicity grade ≥3, and neutropenia (ANC<0.5x109/L) was 42%(19/45), 4.4%(2/45), and 24.4%(11/45) in Cycle-1, and 44%(15/34), 8.8%(3/34), and 41.2% (14/34) in Cycle-2. All CRS were of grade 1-2. One patient experienced grade 5 neurotoxicity. Seventeen received allogeneic HSCT. Thirteen patients relapsed post-blinatumomab at a median 11 months (6.7,16.4) interval between Cycle-1 blinatumomab and relapse. Three relapsed during Cycle-1: two at CNS and one at marrow. At a median follow-up of 18.5 months (9.7, 33.8), 34/45 (76%) are alive and in continued complete remission.

Conclusion: Blinatumomab demonstrated high MRD-clearance with a manageable toxicity profile in a real-world setting. We acknowledge the Blincyto

Humanitarian Access Program for donating Blinatumomab to the patients.

Abstract 202

Pragmatic Protocol Adaptation for High-Risk Pediatric ALL in a Resource-Limited Setting: A Real-World Experience from a Single Center in India

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Purpose: Delivering intensive BFM-based high risk (HR) protocols in low and middle-income countries (LMICs) is often hindered by malnutrition and socioeconomic constraints. At our trust hospital serving patients below the poverty line, 75% of children present with malnutrition. We observed significant challenges in delivering the full 6-cycle HR intensity, leading to a strategic institutional shift toward utilizing the intermediate-risk (IR) protocol for children with high-risk features.

Methods: We retrospectively analyzed 142 children with high-risk ALL as per BFM ALL 95 protocol. Outcomes were compared between patients initiated on the HR protocol (n=32) and those treated with the IR protocol (n=110). Primary endpoints were overall survival (OS), event-free survival (EFS), and cumulative incidence of relapse (CIR). Survival was estimated using the Kaplan-Meier method and compared via log-rank tests.

Results: At a median follow up of 42 months, the 3 year OS and EFS for the whole cohort were 70.1% (61.6–78.5%) and 61.5% (52.5–70.5%). The 3-year CIR was 31.3% (22.3–40.3%). HR-group patients completed a mean of only 4.4 out of 6 intended chemotherapy cycles. The IR protocol demonstrated significantly superior 3-year EFS compared to the HR protocol (65.9% [95% CI: 55.9–75.8%] vs. 45.9% [95% CI: 26.1–65.7%]; p=0.046). The 3-year OS was 72.2% (95% CI: 62.8–81.6%) for the IR protocol vs. 62.1% (95% CI: 43.4–80.8%) for the HR protocol (p=0.100). Treatment-related mortality was 3.5%. Following relapse, 65.8% of patients opted for palliative care.

Conclusion: In settings characterized by malnutrition and resource limitations, the intensive BFM-HR protocol is difficult to complete and associated with high morbidity. Transitioning to the IR protocol significantly improved event-free survival. These findings suggest that for high-risk ALL in LMICs, an adaptable, "completable" intermediate-intensity protocol may be superior to higher-intensity regimens frequently compromised by toxicity.

Abstract 203

Venetoclax as Effective Cytoreduction therapy in Pediatric AML: A Retrospective Study

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Purpose: Hyperleukocytosis can be life-threatening in newly diagnosed acute myeloid leukemia(AML), and requires prompt cytoreduction.

Methods: This retrospective study evaluated venetoclax versus traditional cytoreductive therapy in 113 pediatric AML patients from January 2021 to December 2025, including 40 with acute promyelocytic leukemia (APL).

Results: In the APL group(23 venetoclax, 17 traditional), venetoclax resulted in significantly lower peak white blood cell (WBC) counts after administration of all-transretinoic acid(ATRA) (13.35×109/L vs. 62.22×109/L, P=0.001) and faster WBC reduction to 5×109/L(3 days vs. 12 days, P=0.00), with fewer cases interrupting the treatment of ATAR(21.74% versus 52.94%, P=0.04). Differentiation syndrome incidence (43.48% vs. 64.71%, P=0.18) and remission rates on day 28(100% vs. 94.12%, P=0.43) showed no significant differences. In the non-APL group(n=73), venetoclax similarly achieved faster WBC reduction to <10×10⁹/L (2.5 days vs. 5 days, P=0.00). The remission rates on day 28 of induction in venetoclax and traditional therapy were 91.67% and 75.41%, respectively(P=0.39). In either the APL group or the non-APL group, there were no significant differences in platelet and plasma transfusion doses between venetoclax and traditional cytoreduction. No deaths occurred in APL patients. In the non-APL group, no venetoclax treated patients died, while four traditional therapy patients died from hemorrhage during induction and six from infections or relapse in post treatment.

Conclusion: Venetoclax appears to be an effective cytoreductive option for pediatric AML, particularly in APL.

Abstract 204

JPLSG ALL-R19-BLIN: A phase I/II study of induction therapy with low-intensity cytoreduction prior to blinatumomab for pediatric and AYA R/R ALL reveals tolerability of low-dose cytarabine and clinical biomarkers

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Purpose: Blinatumomab has demonstrated efficacy for relapsed/refractory (R/R) ALL. While it is preferable to reduce leukemia burden before the treatment, strong chemotherapy is often not feasible due to prior treatments or comorbidities. Thus, gentler cytoreduction regimens are needed. Also, identifying biomarkers is necessary to predict and enhance treatment efficacy.

Methods: Pediatric and AYA patients (≤25 years) with R/R B-ALL received continuous infusion of cytarabine at 20 mg/m²/day for 7 days, followed by two cycles of blinatumomab. Comprehensive analyses of treatment response, immune microenvironment in bone marrow (BM) and peripheral blood (PB), and gut microbiota and metabolites were performed to identify biomarkers. This phase I/II study was approved by the National Hospital Organization Review Board for Clinical Trials (Nagoya) and registered with jRCT (jRCTs041210107).

Results: Among the 20 evaluable patients, the overall response rate was 50%, consistent with prior studies. Notably, TP2 BM assessment (day 15 of cycle 1) showed a significant association with subsequent achievement of CR at the end of 2 cycles of blinatumomab, with blast percentages of 0% (range, 0–0%) in the CR group and 58.0% (range, 43.8–84.6%) in the non-CR group ($P < 0.01$). In the mass cytometry analysis, a higher number of absolute CD3+ T cell count in PB at TP2 and TP3 (the end of cycle 1) was observed in CR patients. Furthermore, CR patients maintained more naïve and memory CD4+ T cells. Gut microbiota analysis revealed that CR patients had an abundance of bacteria that induce metabolism favorable to immune activation, while the microbiota of non-CR patients showed a bias towards certain bacteria.

Conclusion: Low-dose cytarabine cytoreduction followed by blinatumomab is well tolerated as a treatment of R/R pediatric B-ALL, particularly for patients with prior intensive treatments or comorbidities. Integrated immunological and microbiome analyses provide novel biomarkers which lead to improving blinatumomab efficacy.

Abstract 205

T-cell markers expression as basis of immuno-oriented treatment protocol for anasplastic large cell lymphoma.

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Purpose: Anaplastic large cell lymphoma (ALCL) is a rare subtype of non-Hodgkin lymphoma characterized by various morphological variants and CD30, ALK expression. This study aimed to evaluate the efficacy of immuno-oriented protocol ALCL NII DOiG 2003 for primary diagnosed ALCL.

Methods: Between 1993 and 2026, 113 patients with newly diagnosed ALCL were included in cohort study. The median age was 12 years, and the median follow-up was 111 months. Patients received standard NHL-BFM 95 (n=52) or ALCL NII DOiG 2003 (n=61); most presented with advanced stages.

Results: Overall survival (OS) in patients treated with ALCL NII DOiG 2003 protocol (90.8%) was higher, compared with NHL-BFM 95 protocol - 86.5% (p=0.4). EFS rates were 91.4% vs 70.1% (p=0.04), and relapse-free survival (RFS) rates were % 94.6 vs 76.2 %, respectively (p=0.012).

Conclusion: The use of a differentiated, immuno-oriented treatment approach for ALCL that takes into account T-cell markers expression appears to be more effective and is associated with improved survival outcomes.

Abstract 206

Quality of Life Analysis of Survivors of Pediatric Acute Lymphoblastic Leukemia treated with BFM ALL Protocol

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Purpose: Acute Lymphoblastic Leukemia (ALL) is the most common childhood cancer worldwide and with treatment advances, prospects of survival have markedly increased. As these children move from treatment to survivorship they may be vulnerable for various late effects which can put them at risk for a poor health related quality of life. The objectives were to assess HRQOL of pediatric ALL survivors and its relationship with use of cranial irradiation.

Methods: This cross sectional study included 70 pediatric ALL survivors and 70 parents of the same children (N = 140). The study evaluated the HRQOL of ALL survivors using PedsQLTM4.0 generic core scales. Higher scores indicate better HRQOL.

Results: Mean age of children at analysis and diagnosis were 11.17 years and 5.9 years respectively. The mean and SD of children's overall HRQOL score was (94.3+12.5) and no children had low HRQOL.As per child self-report, the lowest mean sub-scale HRQOL score was in School functioning (83.0 + 30) whereas the highest mean sub-scale score was in Physical functioning (96.7+12.3). Significant difference could not be found among HRQOL scores and age at diagnosis and cranial irradiation. HRQOL scores of child self-reports and parent proxy reports indicated moderate but significant correlations for the domains of (psychological health: r = 0.306, p = 0.05; total HRQOL score: r = 0.246, p = 0.05; emotional functioning: r = 0.423, p = 0.01; school functioning: r = 0.80, p = 0.01). Regression analysis revealed that homemaker-mothers had a significantly positive impact on Physical functioning(R2 =0.372 , t=-3.088, p=0.003),Emotional functioning(R2 =0.387, t=-2.462,p=0.017) and Social functioning(R2 =0.365,t=-3.019,p=0.004).Rural residence has a positive impact on Physical(R2 =0.372,t=-3.086,p=0.003) and Emotional (R2 =0.387, t=-3.232,p=0.002) functioning.

Conclusion: ALL survivors had high HRQOL. Low score in School Functioning may indicate presence of neurocognitive deficits and the need for psychosocial support in the form of cognitive remediation training.

Abstract 207

Structural Chromosomal Abnormalities Adversely Impact Prognosis in Otherwise Favorable High-hyperdiploidy Pediatric B-Cell Acute Lymphoblastic Leukemia

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Purpose: High hyperdiploidy (HeH) confers favorable prognosis in childhood ALL, but relapse cases persist. Whether additional structural abnormalities (SCAs) elevate risk within this otherwise favorable group remains debated.

Methods: We analyzed 375 children with HeH-ALL.Patients were divided into HeH-SCA (n=197) and HeH-only (n=178) groups. Clinical characteristics, event-free survival (EFS), overall survival (OS), and relapse-free survival (RFS) were compared. Cox regression identified prognostic factors for RFS.

Results: The two groups were well-matched in terms of age, sex, initial white blood cell count, and risk stratification (all P>0.05).

Conclusion: After a median follow-up of 63.6 months (95% CI: 61.63-65.56), patients with HeH-SCA demonstrated significantly inferior 5-year EFS (89.1% ± 2.3% vs. 95.7% ± 1.6%, P=0.036) and 5-year RFS (91.0% ± 2.2% vs. 97.3% ± 1.3%, P=0.024) compared to the HeH-only group. The survival curves diverged early during follow-up. However, 5-year OS was excellent and comparable between groups (98.0% ± 1.0% vs. 98.3% ± 1.0%, P=0.898).Notably, among relapsed patients in the HeH-SCA group, 35.3% (6/17) had multiple structural abnormalities.In multivariate analysis, SCAs (HR=2.81, 95% CI: 1.02-7.71, P=0.044) and adverse age (HR=4.24, 95% CI: 1.23-14.64, P=0.022) remained independent predictors of inferior RFS, indicating that SCAs conferred a 2.81-fold increased relapse risk. Conclusions: Chromosomal structural abnormalities, particularly when multiple, define a distinct subgroup within prognostically favorable HeH-ALL that carries a higher relapse risk. This prognostic heterogeneity suggests that incorporating structural abnormality status—especially multiplicity—into risk stratification could optimize treatment and monitoring strategies.

Abstract 208

Risk and response adapted treatment based on cytokine and clinical condition in pediatric hemophagocytic lymphohistiocytosis: A multicenter prospective study

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Purpose: HLH is rapidly fatal, requiring timely treatment. Aggressive initial therapy may increase toxicity. This study explored pediatric HLH stratification and treatment based on cytokine levels and clinical condition.

Methods: 106 pediatric HLH from 15 Chinese hospitals (09/2022-10/2025) were stratified into 4 risk groups by IL-10/IFN-γ/clinical severity. Lower-risk patients received dexamethasone(Dex)/ruxolitinib(Rux) monotherapy; higher-risk received HLH-94±Rux. Treatment was adjusted per response.

Results: 31 patients (29.1%) attained CR with monotherapy.12-month OS was 85.8% , EFS was 73.5%. Low-risk and intermediate-risk non-critical patients had significantly better survival than high-risk and intermediate-risk critical patients (P<0.05). Dex and Rux showed comparable efficacy in lower-risk groups.

Conclusion: Risk-stratified therapy based on cytokines/clinical severity enabled precise HLH management, avoiding chemotherapy overtreatment and improving survival. Dex and Rux had comparable efficacy in lower-risk patients. Close monitoring of intermediate-risk non-critical patients is essential.

Abstract 209

The Value of Minimal Residual Disease-Guided Risk Stratification in Different Subtypes of Pediatric B-Cell Acute Lymphoblastic Leukemia

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Purpose: To evaluate the clinical value of minimal residual disease (MRD)-guided risk stratification in pediatric patients with B-cell acute lymphoblastic leukemia (B-ALL).

Methods: This was a retrospective cohort study. A total of 666 newly diagnosed B-ALL children between October 2018 and June 2023 were enrolled. Risk stratification and treatment were conducted according to the ZJCH-ALL-2019 protocol.

Results: Risk stratification was adjusted based on MRD levels on day 15 and day 29 in 153 (23.0%) and 5 (0.8%) patients, respectively. The 5-year OS and EFS rates were 95.9% and 92.1%, respectively. Multivariate analysis identified MRD on day 15 and day 29 as independent adverse prognostic factors.

Conclusion: In patients with ETV6::RUNX1 positivity or hyperdiploidy, MRD is less effective in distinguishing between low-risk and intermediate-risk groups but can precisely identify the high-risk group with significantly poorer prognosis. Conversely, in other subtypes, MRD effectively stratifies patients into low, intermediate, and high-risk categories, demonstrating superior discriminatory power in prognostic stratification.

Abstract 210

NK-cell therapy after allogenic hematopoietic stem cell transplantation in patients with acute leukemias

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Purpose: Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is an effective and sometimes the only life-saving method for treating various hematological diseases, both malignant and non-malignant. However, relapses and post-transplant complications, such as graft-versus-host disease (GvHD) and infections, remain significant and unresolved problems. NK-cell therapy is a

promising approach not only to potentiate the antitumor immune response, but also to improve treatment outcomes after allo-HSCT. To evaluate the safety and efficacy of allogeneic NK-cell-based therapy in patients with relapsed and refractory acute leukemias.

Methods: 25 patients since 1 to 18 years diagnosed with acute lymphoblastic (ALL) and acute myeloid leukemia (AML), refractory to therapy, received allo-HSCT from fully match donor followed by infusion of allogeneic NK-cells as immunotherapy. The primary endpoints were safety and successful expansion of donor NK-cells, defined as >100 donor NK cells/μL peripheral blood at day 14 after NK-cell infusion [(absolute lymphocyte count/μL) × (% of lymphocytes that are CD56+/CD3− NK-cells) × (% of donor chimerism using standard short tandem repeat testing)]. Secondary endpoints included response assessment according to IWG 2006 ALL or IWG AML.

Results: NK-cell infusions were well tolerated, no side effects were observed in the presented patients. Cytological remission of AML and ALL were achieved, and minimal disseminated disease was negative found. It is worth noting that, despite the absence of systemic IL-2 administration, an increase in activated Ki-67+CD127−FoxP3+CD25hiCD4+ Treg cells of the recipient was observed after NK-cell therapy.

Conclusion: The introduction of donor NK cells after allo-HSCT in patients with relapsed and refractory forms of acute leukemia allows activating additional functions of antitumor immune surveillance and hopes for increasing the effectiveness of therapy for unfavorable variants of acute leukemia, due to the expansion of the possibilities of cellular therapy strategies.

Abstract 211

Allogeneic Hematopoietic Stem Cell Transplantation in Infants with Acute Leukemia: A Single Center Experience in 18 Patients

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Purpose: Infant leukemia (acute leukemia in children <1 year) is rare, comprising up to 5 % of pediatric acute leukemias (AL) cases. It is difficult to treat, so specialists worldwide are developing dedicated protocols for these patients.

Methods: Eighteen patients with infant leukemia (IL) underwent allo HSCT (Jan 2021-Dec 2024) at the Lev Durnov Institute (Nikolay Blokhin National Center of Oncology). Median age - 5.7 y.o. (0-11 months). M/F=8/10. The cohort comprised 9 AML cases (50%), 7 ALL cases (27.7%), and 2 mixed phenotype acute leukemia cases (22.3%). KMT2A rearrangements occurred in 33.3% of patients (n=6). Donors: haploidentical - 7 (38.8%), match related donor (MRD) - 5 (27.8 %), matched unrelated donor (MUD) -6 (33.4 %). Stem cell source: PBSC - 83.4%, BM - 16.6%. Graft manipulation: haploidentical -3 pts. underwent TCRαβ/CD19-depletion and 4 - PtCy. Conditioning regimens: ALL - treosulfan –based in 4, busulphan-based in 3 pts; AML/ABiL – treosulfan/thiotepa (n=6) or treosulfan/melphalan (n=5) based. IST therapy: haplo with TCR a/b/CD19 depletion: tocilizumab+abatacept, PtCy – CsA/ruxolotinib, abatacept+vedolizumab, MRD - CsA/ruxolotinib, abatacept, MUD – tacro/ruxolotinib, ATG, abatacept.

Results: Median of follow-up of 36 months (48 -24). All pts. but two engrafted. Nine are alive after allo-HSCT without relapse. OS propability is 50 %, in the group of patients with KMT2A- 66% (n = 4). Median of ANC recovery was 13.6 (10.3-22.6). The relapse rate after allo-HSCT was 27.7%. Cumulative incidence for acute GvHD of 1-2 gr. was 47.3%, 3-4 gr. was only 3.5%. Chronic GvHD was found in 4 patients, 2- extensive form.

Conclusion: Our center’s experience indicates acceptable transplant related mortality and satisfactory relapse rates following allo HSCT in patients with infant leukemia. Considering the unique characteristics of this age group, careful dose selection of chemotherapy agents and a balanced assessment of indications for allo HSCT are imperative.

Abstract 212

Post Transplant Lymphoproliferative Disorder (PTLD) in Pediatric Liver Transplant (LT) recipients: A single centre experience from a tertiary care centre from South India

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Purpose: Post transplant lymphoproliferative disorder (PTLD) is a serious and potentially life-threatening complication following solid organ transplantation, particularly in pediatric liver transplant (LT) recipients. Despite advances in immunosuppressive strategies and EBV monitoring, PTLD continues to contribute significantly to morbidity and mortality. This study aimed to analyse the clinical profile, histopathological characteristics, and outcomes of children diagnosed with PTLD after LT.

Methods: We conducted a retrospective study of children aged <18 years who underwent LT between January 2011 and December 2025 at a single tertiary care centre. Patients with histologically proven PTLD were included for analysis.

Results: Among 910 pediatric LT recipients, 17 (1.87%) developed PTLD. Median interval from transplantation to PTLD diagnosis was 22 months. Early-onset PTLD (<1 year) occurred in 35.2%. Multiorgan involvement is seen in 11 patients (64.7%). Lymph node involvement was the most common presentation (58.8%) followed by gastrointestinal involvement (41.2%). Histopathological revealed hyperplasia changes in 6 (35.2%), polymorphic PTLD in 3 (17.7%) and lymphoma in 8 (47.1%), including 3 Diffuse large B-cell lymphoma and 1 Burkitt lymphoma. EBV association was observed in 88.2% (EBER ISH positive), and CD20 positivity in 94.1%. Treatment included reduction of immunosuppression alone in 2 patients or combined with rituximab in 8 patients and/or chemotherapy in 7 patients. Complete response was achieved in 15 patients while 2 patients died.

Conclusion: PTLD following pediatric LT was predominantly EBV driven and frequently involve multiple sites. Early detection and stepwise management with reduction of immunosuppression and rituximab and/or chemotherapy resulted in high remission rates. The lower incidence in our study cohort reflects lower immunosuppression burden and effective surveillance.

03. OCULAR ONCOLOGY — POSTER

Abstract 213

Group A Retinoblastoma: A study of two cases

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Purpose: The purpose was to study the clinical features, treatment, and outcome of retinoblastoma (RB) in Mongolia.

Methods: This was a retrospective study of two patients with RB detected in the 2nd month of life and 28 months of age.

Results: The mean age at diagnosis of RB was 15 months (mean, 15 months; range, 2–28 months). There were two male with bilateral RB. Two patients were brought in with complaints of leukocoria and had an intraocular tumor at presentation. Based on the International Classification of Intraocular Retinoblastoma, the tumors were classified as Group A (n = 2), Group D (n = 2). Macular involvement was noted in 2 (50%) eyes. The primary treatment included systemic chemotherapy without focal treatment in those patients and second treatment included systemic chemotherapy with green laser treatment. Of the two patients who received 6 times systemic chemotherapy with laser treatment, globe salvage was achieved in 4 (100%) eyes over a mean follow-up period of 7 months (mean, 3 months; range, 4–7 months).

Conclusion: Group A RB, if detected early, has a favorable outcome of ocular and life salvage.

Abstract 214

Cavitary Retinoblastoma: Clinical spectrum, imaging characteristics, and outcomes from a tertiary care center in Pakistan

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Purpose: Cavitary retinoblastoma is a rare phenotypic variant characterized by intra tumoral cavities and distinctive imaging features. Recognition is important as its behavior may differ from typical retinoblastoma. This study describes its clinical features, imaging findings, and treatment outcomes

Methods: A retrospective descriptive study was conducted from July 2024 to December 2025 at a tertiary care centre. A total of 90 retinoblastoma patients, including newly diagnosed and follow-up cases, were reviewed. Cavitary retinoblastoma was diagnosed based on characteristic clinical appearance and imaging findings on ultrasound B-scan, Optical Coherence Tomography (OCT), and/or magnetic resonance imaging (MRI). Demographic data, laterality, treatment modalities, and clinical outcomes were analyzed using descriptive statistics.

Results: Seventeen patients were identified with cavitary retinoblastoma. The diagnosis was established clinically and supported by images showing intra tumoral cavitations without calcification or with atypical reflectivity patterns. Most patients were managed conservatively with systemic chemotherapy and focal therapies. Tumor regression with persistence of cavitary architecture was observed in the majority of cases. Globe salvage was achieved in 86% treated eyes, and no extraocular extension was noted during the follow-up period.

Conclusion: Cavitary retinoblastoma represents a rare but distinct phenotype with characteristic clinical and imaging features. Awareness of this entity is essential to avoid misdiagnosis and unnecessary aggressive treatment. Conservative management with close monitoring can result in favorable outcomes and high globe salvage rates

Abstract 215

Clinical Analysis of Autologous Stem Cell Transplantation in the Treatment of 16 Cases of Stage IV Retinoblastoma

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Purpose: To evaluate the efficacy and safety of autologous hematopoietic stem cell transplantation (AHSCT) in children with stage IV retinoblastoma.

Methods: Clinical data of 16 patients diagnosed with stage IV retinoblastoma who received AHSCT at our hospital from July 2020 to May 2025 were retrospectively analyzed. The primary endpoints were 1-year event-free survival (EFS) and overall survival (OS). Secondary endpoints included transplant-related toxicity, hematopoietic reconstitution, and complications.

Results: Among the 16 patients with stage IV retinoblastoma, 11 were male and 5 were female; 12 had unilateral disease and 4 had bilateral disease; 7 were stage IVa and 9 were stage IVb. The median age at diagnosis was 21 months (range: 0.07–49 months). All 16 patients received conventional chemotherapy and underwent peripheral blood autologous hematopoietic stem cell collection before transplantation. Myeloablative conditioning regimens based on thiotepa, carboplatin, and etoposide were administered, with dose adjustments according to individual conditions. All patients successfully completed autologous peripheral blood stem cell infusion.The median time to neutrophil engraftment was 9 days (range: 8–11 days), and the median time to platelet engraftment was 11 days (range: 8–21 days).Twelve patients survived and 4 died (3 with stage IVb, 1 with stage IVa), with no transplant-related deaths.The median overall survival was 33 months (range: 1–63 months): 46 months for stage IVa (range: 1–63 months) and 25 months for stage IVb (range: 4–38 months).The 1-year EFS and OS rates were 68.9% and 87.5%, respectively. For stage IVa, the 1-year EFS was 85.7% and OS 85.7%; for stage IVb, the 1-year EFS was 41.5% and OS 88.9%.Common adverse events included grade IV myelosuppression, grade II–III oral mucositis, and grade III gastrointestinal toxicity. No significant cardiopulmonary, neurological, or ototoxicity was observed.

Conclusion: 1.Autologous hematopoietic stem cell transplantation is an effective treatment for stage IV retinoblastoma, with reversible side effects, favorable tolerability, and manageable adverse reactions. 2.Patients with stage IVb retinoblastoma with central nervous system metastasis may achieve short-term benefits from AHSCT, but long-term prognosis remains poor. 3.AHSCT can serve as a potentially curative therapeutic modality for stage IVa retinoblastoma without central nervous system metastasis.

Abstract 216

Strengthening Retinoblastoma Care in Pakistan: Development and Implementation of the PSPO Standard-of-Care Treatment Protocol for Pediatric Retinoblastoma (PSPO RB 2022-01)

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Purpose: Improving outcomes in retinoblastoma (RB) requires standardized care and reliable national data systems, particularly in low- and middle-income countries (LMICs). In alignment with the World Health Organization Global Initiative for Childhood Cancer (GICC), the Pakistan Society of Pediatric Oncology (PSPO) initiated the development and implementation of a National Standard-of-Care (SOC) Treatment Protocol for RB in 2019, based on national and international guidelines. This study aimed to standardize treatment practices, strengthen data systems, improve patient outcomes nationally, and describe the implementation process as a model for other LMICs.

Methods: A multidisciplinary team was formed by engaging all pediatric oncology centers treating RB and the Pakistan Society of Pediatric Ophthalmology to identify ophthalmology experts. Representatives from radiology, pathology, radiation oncology, and pharmacy were also included, along with an epidemiologist and biostatistician. Structured national online meetings were conducted to review international evidence and local experience. A WhatsApp group facilitated communication and consensus building. Through an iterative consultative process, consensus-based National RB SOC guidelines were developed and endorsed, with additional input from national conferences. A REDCap-based database and standardized case report forms were used for data collection. Following national and institutional ethical approvals, virtual training was provided to data managers. A centralized monitoring system with routine quality checks, audits, and feedback ensured data accuracy. Workshops were conducted to improve protocol adherence and expand participation.

Results: The protocol was implemented across Pakistan in mid-2022. Eleven centers participated, enrolling over 400 patients. Standardized treatment and structured data systems improved collaboration, consistency of care, and enabled national outcome evaluation.

Conclusion: Development of national SOC guidelines for treatment of RB, has significantly improved the infrastructure and feasibility in a resource constraint setting like Pakistan. This model provides a scalable framework for improving retinoblastoma outcomes and may serve as a template for other pediatric oncology programs in similar contexts.

04. NEURO-ONCOLOGY — ORAL PRESENTATION

Abstract 217

Intensive complex treatment including tandem high-dose therapy with autologous hemopoietic stem cell transplantation in pediatric patients younger than 4 years diagnosed with rare embryonic CNS tumors

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Purpose: While embryonal central nervous system (CNS) tumors represent about 10% of all CNS lesions in children this population is in turn very heterogenic. Apart from different meduloblastoma (MB) subtypes there are also extremely rare conditions, e.g. atypical teratoid rhabdoid tumor (ATRT), embryonal tumor with multilayered rosettes (ETMR), or primary CNS neuroblastoma (NB). There is also a small subset of agressive embryonal tumors assigned to NOS (non otherwise specified) category. Due to extreme rarity of these conditions there are generally no strict evidence-based treatment guidelines. Although the intensive approach

based on aggressive surgery, chemotherapy and craniospinal iradiation (CSI) seems tob be effective, the latter still leads to severe cognitive impairment in younger (< 4 years) patients. This cohort may benefit from consolidation based on high-dose chemotherapy (HDCT) with autologous hemopoietic stem cell transplantation (auto-HSCT), which is successfully employed in younger patients with MB. We summarize our experience in younger patients with rare embryonal CNS tumors.

Methods: A total of 32 children with a median age of 40 (range 8-48) months diagnosed with rare tumors were included in this study, among them ETMR (n=14), NB (n=2), pineoblastoma (PB, n=5) or NOS (n=11). All patients underwent surgery and 3 courses of chemotherapy (carboplatine-etoposide) with or without intrathecal methotrexate (IT Mtx). Tandem HDCT with auto-HSCT was given to all patients achieving complete (CR, n=16) or partial (PR, n=16) response. Conditioning regimen 1 included carboplatine (500 mg/m2 on D1 to D4) and etoposide (250 mg/m2 on D1 to D4) with IT Mtx. The second one consisted of thiotepa (300 mg/m2 on D1 to D3) and cyclophosphamide (1500 mg/m2 on D1 to D3). The median CD34+ cells count was 3.9 (1.24–8) and 4.4 (1.65–8) × 106/kg on 1st and 2nd transplant, accordingly. Further on, 13 of 32 patients received delayed CSI.

Results: The non-relapse mortality rate was 12% (95% CI 0%-24%). All patients engrafted at a medium of D+10 (5-20) after th 1st and D+14 (9-34) after the 2nd auto-HSCT, accordingly. With a median observation period of 25 (2-133) months the 3-year overall (OS) and event-free (EFS) survival are 48% and 43%, accordingly. The patients achieving CR prior to consolidation showed slightly better OS rate compared to patients achieving PR (54% vs. 34%), although this difference has not reached statistical significance (p=0.3). However, long-term OS was different in patients with different tumor variants (100% for NB, 43% for ETMR, 43% for NOS, and 0% for PB; p= 0.06). The CSI was also a significant factor with OS being significantly higher in CSI recipients (p=0.003).

Conclusion: The dose-intensive consolidations may be a feasible option allowing a delayed CSI in younger children in order to potentially mitigate long-term treatment effects. The overall results are, however, still to be improved, which requires multicenter communication in context of these extremely rare conditions.

Abstract 218

Clinical features and prognosis of pediatric intracranial malignant germ cell tumors at a single institution

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Purpose: Pediatric intracranial malignant germ cell tumors (iMGCTs) are diverse pediatric brain cancers that may be classified as germinomas or non-germinomatous germ cell tumors (NGGCTs). This study aimed to evaluate the clinical characteristics and prognosis of iMGCTs at a single institution.

Methods: A retrospective analysis was conducted on the clinical data of patients with pediatric primary iMGCTs treated at the Medical Oncology Department, Beijing Children's Hospital from May 2017 to December 2024.

Results: Thirty-six patients were enrolled. The time from symptom onset to diagnosis was 0.2–40 months. Based on histopathology and tumor markers, 9 cases (25%) were diagnosed as germinomas, 26 (72.2%) as NGGCTs, and 1 as unclassifiable. All patients received chemotherapy, radiotherapy, and surgery. For patients with germinomas, 3-year and 5-year overall survival (OS) were each 100%, and 3-year and 5-year event-free survival (EFS) were 100% and 88.9%, respectively. For patients with NGGCTs, 3-year and 5-year OS were 95.8% and 91.3%, respectively, and 3-year and 5-year EFS were each 69.2%. Patients with NGGCTs who underwent surgical tumor resection during first-line treatment had significantly higher EFS than those who did not (88.2% vs. 33.3%; P=0.002). Patients with NGGCTs who underwent surgical tumor resection during first-line treatment had significantly higher EFS than those who did not (88.2% vs. 33.3%; P=0.002). The presence of choriocarcinoma components was statistically significantly associated with worse EFS (50% vs. 92.9%; P = 0.039).

Conclusion: Even with multimodal treatment, the EFS for patients with NGGCTs remains suboptimal. Choriocarcinoma and the absence of surgical resection during first-line therapy are poor prognostic factors, highlighting the potential importance of surgery in NGGCT management and enhanced chemotherapy intensity for high-risk disease.

Abstract 219

DELAYED DIAGNOSIS OF PEDIATRIC CNS TUMORS: THE UNSOLVED PROBLEM IN THE MOLECULAR ERA.

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Purpose: Tumors of the Central nervous system (CNS) are the most common solid malignancies in children and adolescents, and the leading cause of cancer-related mortality in this age group. The symptoms of CNS tumors are often non-specific, which frequently leads to delayed diagnosis. The current study aims to evaluate the pattern of delayed diagnosis in a developing country and suggest a universal model for solving the problem.

Methods: We have collected data from pediatric patients (< 18 years) treated in the neurosurgery department, three major chemotherapy clinics, and the radiation therapy department from 1st January 1995 to 31st December 2020 in Armenia. Patients' characteristics, tumor characteristics, and time from first symptoms to diagnosis were calculated using descriptive statistics.

Results: The multicenter data analysis revealed 142 children and adolescents diagnosed with primary CNS tumors over 26 years. Among them 79 (55.63%) were males. The median age at diagnosis was 84 months (range, 3-204 months). Medulloblastomas and other embryonal tumors, low-grade gliomas, and high-grade gliomas accounted for 31.69%, 19%, and 9.15% of all cases, respectively. For 26.79% of cases, histological diagnosis was not available. The mean time from the first symptoms to diagnosis was 5.95 months and the median time was 1 month (range, 0.2-70 months). The mean and median times from the first complaint to the diagnosis of medulloblastomas and other embryonal tumors, low-grade gliomas, and high-grade gliomas accounted for 2.13 months and 1 month (range, 0.2-24 months), 12.5 months and 3 months (range, 0.25-70 months), 1.1 months and 1 month (range, 0.25-3 months), respectively.

Conclusion: Delayed diagnosis of pediatric CNS tumors is a major issue in Armenia. A more pronounced delay in diagnosis was observed in patients with low-grade gliomas. We see the continuous training of primary care physicians and multidisciplinary work as a solution to this major problem.

Abstract 220

PEDIATRIC HIGH-GRADE GLIOMAS ASSOCIATED WITH GENETIC TUMOR SYNDROME: A SINGLE-CENTER EXPERIENCE

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Purpose: Pediatric high-grade gliomas (pHGG) arising in the context of genetic tumor syndrome (GTS), including constitutional mismatch repair deficiency (CMMRD) and Li-Fraumeni syndrome (LFS), represent a distinct clinical entity with specific therapeutic challenges.

Methods: We retrospectively analyzed 28 pHGG patients selectively screened for GTS based on phenotypic features and family history. GTS-associated germline pathogenic variants (GPVs) were detected using NGS (custom multigene panel, WGS), Sanger sequencing.

Results: Nine of 28 (32%) pHGG patients (6 female, 3 male) had GTS-associated pHGG. Seven had CMMRD (PMS2 GPVs n=5; MSH2 n=1; MSH6 n=1) and two had LFS due to TP53 GPVs. Median age at diagnosis was 9.8 years (range, 3.9–11). Histology included diffuse HGG (n=6) and a pleomorphic mitotically active tumor with microvascular proliferation without necrosis (n=1); two patients were managed without biopsy, with MRI suggestive of low-grade glioma. Four CMMRD patients developed additional malignancies (hemoblastosis n=3, retinoblastoma n=1). Seven patients underwent surgery (partial/subtotal resection n=6, gross total resection n=1). Seven received radiotherapy; four received concurrent temozolomide. Four patients received immune checkpoint inhibitors (ICI): three with CMMRD (nivolumab and/or pembrolizumab) and one with LFS (nivolumab). One CMMRD patient achieved a transient partial response to ICI

before progression and subsequently died from Listeria monocytogenes meningoencephalitis; three progressed on ICI. At a median follow-up of 23.6 months, six patients had died. All three survivors had PMS2-deficient CMMRD: one is alive 7.7 years after chemoradiotherapy for a histologically unverified high-grade tumor, and two are under observation without surgery or oncologic treatment at 7.5 and 2.7 years, respectively.

Conclusion: GTS-associated pHGG carry a poor prognosis despite multimodal therapy, and ICI provided only transient benefit in CMMRD patients without durable disease control. The observation that all long-term survivors had PMS2-deficient CMMRD, including two managed with observation alone, suggests that a subset of CMMRD-associated CNS tumors may follow a more indolent course.

Abstract 221

Improved Survival of Paediatric High Grade Glioma in the Modern Era

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Purpose: To evaluate outcomes of paediatric high-grade glioma (HGG) in Hong Kong, comparing treatment eras before and after the establishment of the Hong Kong Children's Hospital in 2019.

Methods: A retrospective review of 68 paediatric HGG patients diagnosed from 1999 – 2025 was performed. Patients were stratified by diagnostic era: pre-2019 (n=51) and ≥ 2019 (n=17). Overall survival, molecular testing, use of targeted therapy and extent of surgery were analysed.

Results: Median overall survival improved in the recent cohort: 1.06 years (range: 0.02-21.44) for patients diagnosed before 2019, compared with 1.82 years (range: 0.01-6.51) in post-2019 cohort, a statistical significant difference (log-rank test p = 0.02). Use of molecular diagnostics increased from 18% (9/51) before 2019 to 100% (17/17) thereafter. Targeted therapy was administered to 4% (2/51) of pre-2019 patients, compared with 65% (11/17) post-2019. Among treated patients, the median survival was longer (1.88 vs 1.06 years) with a significant benefit in survival (log-rank p = 0.007). Infant subtypes, including Infant glioblastoma and infant hemispheric glioma (n=7) showed more favourable outcome, with 71% (5/7) achieving long-term survival. Two patients receiving molecular matched targeted therapies had exceptional survival: one with BRAF V600E–mutant gliosarcoma and an acquired PIK3CA mutation survived 5.6 years, and another with PDGFRA amplified glioblastoma survived 3.8 years. Extent of resection did not correlate with improved survival, likely reflecting underlying tumour biology and anatomical constraints.

Conclusion: Survival outcomes for paediatric HGG have improved in the modern era, driven by widespread adoption of molecular diagnostics and increased use of targeted therapies. These findings highlight the growing impact of precision medicine in this population.

05. NEURO-ONCOLOGY — POSTER

Abstract 222

Epidemiology and Therapeutic Outcomes of Pediatric Medulloblastoma in Low- and Middle-Income Countries: A Systematic Review and Meta-Analysis

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Purpose: Medulloblastoma is the most common malignant brain tumor in children, but outcomes still vary across health systems. While survival has improved with multimodal therapy in high-income countries, children in low- and middle-income countries (LMICs) continue to face barriers, including delayed diagnosis and limited access to adequate therapy. This study aims to summarize the epidemiology, treatment patterns, and outcomes of pediatric medulloblastoma in LMICs.

Methods: This study adhered to the PRIMSA 2020 statement. All studies with epidemiological and therapeutic data on medulloblastoma in children were included. A random-effect single-arm meta-analysis model was selected. The incidence of medulloblastoma in LMICs was estimated. Thus, the treatment trends and the event-free survival (EFS) were reported.

Results: A total of 44 studies were included, yielding 2,779 medulloblastoma cases across 23 LMICs. In sum, the incidence among LMICs was 197.53 cases per 1,000 pediatric brain tumor cases (95%CI 163.04 – 237.27) with a higher incidence in the Asia region. Surgery was done in 99.97% cases, while radiotherapy and chemotherapy were given in 96.65% and 59.26% cases, respectively. The 5-year EFS among LMICs was estimated to 51.86% (95%CI 34.65 – 68.65%), with the European country (Serbia) having the highest 5-year EFS (62.80%; 95%CI 52.22 – 72.28%), and Asia having the lowest (50.06%; 95%CI 21.22 – 78.85%).

Conclusion: The incidence of pediatric medulloblastoma among LMICs is high. With a significant barrier, however, most cases receive adequate therapy, although only half of the patients receive chemotherapy. Nonetheless, improvement of medulloblastoma therapy in LMICs is needed to achieve a better 5-year EFS.

Abstract 223

A rare central nervous system (CNS) tumor with HGNET-MN1 alteration in a child, a clinical case

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Purpose: Astroblastoma (AB) is one of the most rare pediatric CNS tumors comprising 0.45-2.8% of cases. In spite of having a characteristical morphological pattern, astroblastic pseudorosettes, it may be quite heterogeneous, which requires a confirmation by genetis assays aimed at specific aberrations. Whith current diagnosis being based on molecular methods, AB is defined acc. to 2021 WHO classification as a separate MN1-altered entity member (comprised of gliomas, glioneuronal and neural tumors). The clinical course and treatment response may also vary across the AB cohort, which may require different treatment approaches. We here peovide a case report of a child with this rare tumor.

Methods: At an age of 3 years a child developed uncontrolled eye movements and blinking, had headaches. The brain MRI without contrast enhancement (CE) revealed a 31x32x33 mm lesion in posterior part of the left occipital lobe. A subtotal tumor resection was performed. According to post-surgery investigation, there was a post-intervention cyst with borderline contrast accumulation, but no signs of metastases (R+M0 stage). The tumor ha characteristic AB morphology, the reference center also described a high-grade neuroepithelial tumor with MN1 alteration. Given a high-grade morphology and aggressive clinical course of the tumor, 3 chemotherapy (CT) cycles (carboplatin and etoposide) acc. to HIT-2017 guidelines for embryonal tumors were then provided. Although the MRI was unchanged, there were no signs of abnormal metabolic activity post-treatment acc. to 11C-methionine PET/CT. The patient the received intensive consolidation with tandem high-dose chemotherapy (HDCT) with autologous hemopoietic stem cell transplantation (auto-HSCT). The 1st HDCT regimen consisted of carboplatin (500 mg/m2 on Д1 to D4) and etoposide (250 mg/m2 on D1 to D4), the 2nd one of thiotepa (300 mg/m2 on D1 to D3) and cyclophosphamide (1500 mg/m2 on D1 to D3). The engraftment was registered on D+15 and D+13 after 1st and 2nd transplant, accordingly. The Gr 3 hepatic toxicity and Gr 3 oral mucositis were registered after the 2nd transplant. While there were still persistent MRO changes, the patient then received proton-beam craniospinal irradiation (24 Gy) with boost to tumor bed up to 54.6 Gy. All subsequent MRIs show no signs of disease With a current follow-up of 6 years there are no signs of disease progression

Results: An additional molecular test (DNA methylation profiling) allowed timely diagnosis and effective therapy intervention in a rare HGNET-MN1 altered tumor.

Conclusion: AB diagnosis is hardly possible without DNA methylation profiling. Its treatment is also a complex task to its rarity and clinical course being very different based on tumor grade. The radical tumor resection is preferable. If total resection of a high-grade tumor is unattainable, the approach used for aggressive embryonal tumors (CT, HDCT with auto-HSCT and RT) may be feasible.

Abstract 224

Environmental Determinants of Pediatric Brain Tumors in Resource-Limited Settings: Insights from a Referral Center in Pakistan

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Purpose: Pediatric brain tumors are among the most common solid cancers in children, with significant illness and death globally. In resource-limited settings, the impact of these tumors is influenced by social, environmental, and healthcare factors. Acknowledging the role environmental factors play in pediatric brain tumors is key to developing prevention and intervention strategies to improve outcomes. The study aims to evaluate the spectrum environmental determinants in local populations linked with pediatric brain tumors.

Methods: This cross-sectional prospective study was conducted at Children's Hospital Lahore (September 2024–December 2025) following IRB approval. A structured questionnaire adapted from validated LMIC survey tools was finalized with expert input and pre-tested. Caregivers completed the questionnaires via face-to-face interviews. Descriptive analysis was performed using SPSS 26.

Results: The study encompassed 400 pediatric patients diagnosed with brain tumors, with a mean age of 7.01 years and a median age of 6.50 years; males comprised 61% (244/400) of the cohort, resulting in a male-to-female ratio of 1.6:1. Sixty-seven percent of participating families resided in rural areas, and parental educational levels were varied, with 70% having attained some form of education. Only 10% of fathers were engaged in agricultural professions, while 50% worked as daily wage laborers and the remainder in other occupations. The median number of siblings per family was four; additionally, 17% of children had a history of varicella and measles infection. Consanguinity was noted in 70% of families, with 28% reporting a significant history of malignancy and 11% indicating familial brain tumors. Regarding environmental risk factors, 53% of families utilized wood and manure as fuel, with 33% cooking indoors; pesticide use was reported by 64% of families. Thirty-three percent of fathers were active smokers, and 22% of mothers had hypertension. Water sources included tap water (51%), filtered water (45%), and lakes or wells (5%). Dietary patterns showed that 87% of families primarily consumed vegetables and grains. The mean duration of symptoms prior to diagnosis was 6.11 months, with a median duration of 3.00 months, indicative of extended referral and diagnostic intervals. Tumor distribution revealed supratentorial involvement in 42% of cases, infratentorial in 46%, and brainstem in 10%. Surgical intervention was performed in 43% of patients, and VP shunt placement occurred in 50% to address symptom severity.

Conclusion: Conclusion: Pediatric brain tumors in resource-limited settings are influenced by environmental, social, and healthcare factors. Addressing this issue requires multidisciplinary strategies targeting safer environments, public health, and access to care. Ongoing research is needed to clarify causes and develop interventions for at-risk children worldwide.

Abstract 225

Neurological Recovery in Pediatric Malignant Spinal Cord Compression: Determinants and Outcomes from a Contemporary Cohort

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Purpose: Malignant spinal cord compression (MSCC) is a rare but critical oncologic emergency in children that can lead to permanent neurological impairment if not promptly recognized and treated. Evidence describing clinical characteristics and outcomes in pediatric MSCC remains limited.

Methods: We conducted a retrospective cohort study of children diagnosed with MSCC at a tertiary pediatric oncology center between January 2024 and October 2025. Clinical presentation, tumor characteristics, treatment modalities, neurological recovery, and survival outcomes were analyzed. Factors associated with neurological recovery were evaluated.

Results: Twenty-four patients were included (median age 60 months, range 8–158 months). Tumor types included neuroblastoma (33.3%), sarcomas (33.3%), and hematologic malignancies (25.0%). Paraparesis was the most common presenting motor deficit (75.0%), and bowel or bladder dysfunction occurred in 66.7% of patients. The median duration of symptoms prior to diagnosis was 20 days (IQR 9–33), with a median diagnostic delay of 15 days (IQR 10–16). Corticosteroids were administered in 87.5% of patients. Complete neurological recovery occurred in 66.7% of cases with a median recovery time of 30 days (IQR 10–45). At a median follow-up of 10 months, overall survival was 69.2%. Symptom duration ≤25 days was associated with improved neurological recovery.

Conclusion: Early recognition and rapid multidisciplinary intervention are critical in pediatric MSCC. This series highlights symptom duration as a key determinant of neurological recovery and underscores the importance of timely diagnosis to optimize neurological outcomes in children with spinal cord compression.

Abstract 226

CANCER TESTIS ANTIGEN PROFILING IN PEDIATRIC MEDULLOBLASTOMA HIGHLIGHTS LDHC AS A PROMISING TARGET IN HIGH-RISK TUMORS.

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Purpose: Medulloblastoma is the most commonly diagnosed malignant brain tumor in children, and comprises four major subgroups: WNT, SHH, group 3, and group 4 tumors. Group 3 and group 4 tumors are associated with high metastatic propensity, treatment resistance, and poor outcomes. Current treatment relies on multimodal therapy, often causing short-term toxicity and long-term complications. Therefore, there is a need for more precise therapeutics that selectively target tumor cells while sparing normal tissues. Hence, we investigated the expression of cancer/testis antigens (CTAs) – a group of antigens with restricted expression patterns and oncogenic functions - in pediatric medulloblastoma.

Methods: We analyzed the transcriptomic landscape of 228 CTAs in medulloblastoma tumors compared to normal cerebellum tissues using publicly available datasets from Gene Expression Omnibus (GEO), consisting of WNT (n=19), SHH (n=45), group 3 (n=42), group 4 (n=46), mixed group 3/4 (n=2), and unclassified tumors (n=13). To assess functional relevance, one selected CTA candidate was silenced in medulloblastoma cells, followed by colony formation assays.

Results: CTA-based UMAP analysis clearly segregated normal (n=26) from tumor (n=167) tissues, with distinct clustering of tumor subtypes. A total of 40 CTAs demonstrated significant upregulation (pFDR ≤0.05, abs log2FC>1) in tumors. Subtype analysis revealed common upregulation of 21 CTAs across all subtypes, including well-known CTAs such as PRAME and MAGE-C2. Group 3 and group 4 tumors showed unique upregulation of six CTAs, of which four are testis-restricted, including Lactate Dehydrogenase C (LDHC). Preliminary functional analysis demonstrated that LDHC silencing in the group 3 cell line D-458 significantly reduced tumor cell clonogenic ability.

Conclusion: Our findings highlight CTAs as potential biomarkers and therapeutic targets in pediatric medulloblastoma, revealing both shared and subtype-specific expression patterns. Functional analysis further suggests that LDHC may contribute to tumor clonogenic ability in high-risk medulloblastoma.

Abstract 227

Congenital High-Grade Immature Teratoma of the Central Nervous System: A Rare Fetal Case Report

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Purpose: Congenital intracranial teratomas are rare germ cell tumors that account for a small fraction of pediatric brain tumors but carry a poor prognosis due to their rapid growth and aggressive characters. This report describes an autopsy case of a massive intracranial high-grade immature teratoma detected in late pregnancy, emphasizing the diagnostic challenges and pathological features.

Methods: Intracranial mass was first identified by prenatal ultrasound and subsequently analyzed by MRI. Morphologic diagnosis was confirmed by broad panel of immunohistochemical markers, including CD56, SALL4, Ki-67, S100 and Pan-CK

Results: Prenatal ultrasound at 32 weeks of gestation revealed a large, heterogeneous intracranial mass. MRI identified a midline lesion involving the bilateral lateral ventricles, third ventricle, and upper cerebellar vermis. Macroscopic examination revealed a solid, gray-pink-brown 6.5x6x3.5 mm mass within the midline ventricular area, showing signs of focal brain tissue involvement and partial autolysis. Histological analysis, combined with immunohistochemistry (IHC), confirmed a high-grade immature teratoma. The profile showed strong positivity for CD56 and vimentin, with focal positivity for SALL4, a marker indicative of germ cell origin. The Ki-67 proliferation index was significantly high about 60%, reflecting the tumor's rapid growth potential. The markers S100 and CK-Pan were negative, ruling out certain epithelial and mature neural differentiated lineages. Therefore, tumor exhibited primitive neuroepithelial elements and mesenchymal components.

Conclusion: This case highlights the critical role of advanced prenatal imaging and immunohistochemistry in differentiating pediatric intracranial masses. The high Ki-67 index and immature SALL4-positive components categorize this as a high-grade malignancy. Despite the prenatal detection, the aggressive growth pattern of such immature teratomas often leads to devastating clinical outcomes, necessitating early multidisciplinary management and parental counseling.

Abstract 228

Clinically Feasible Integrated Molecular Stratification Identifies Prognostic Subgroups in Standard-Risk Medulloblastoma

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Purpose: Although several molecular stratification systems for medulloblastoma have been proposed, their implementation is limited. The aim was to develop a clinically feasible molecular stratification algorithm for patients with standard-risk medulloblastoma.

Methods: The study included 134 patients with standard-risk medulloblastoma (age >3-5 years, R0M0, non-anaplastic histology, absence of MYC/MYCN amplification) treated between 2012 and 2024. Molecular subgroup assignment was performed using NanoString nCounter expression profiling (23-gene panel). Mutation assessment was performed only in WNT (n=33) and SHH (n=17) subgroups. All patients received craniospinal irradiation at a dose of 23.4–35.2 Gy with a posterior fossa boost to 54–55 Gy followed by maintenance chemotherapy.

Results: The distribution of patients across molecular subgroups was as follows: WNT (n=41), SHH (n=20), Group 3 (n=23), and Group 4 (n=50). WNT tumors demonstrated the most favorable outcomes, with 5-year overall survival (OS) of 93% and event-free survival (EFS) of 84%. In contrast, survival was lower in the SHH and Group 3 (OS 77% and 74%; EFS 60% and 63%, respectively). Based on expression profiling, tumors within the non-WNT/non-SHH cohort were further stratified into three transcriptional subtypes: GR3-pure, GR4-pure, and GR3/4. The poorest 4-year EFS was observed in the GR3-pure (67%) and GR3/4 (57%) subtypes, whereas the GR4-pure subtype was associated with a favorable outcome (87%, p=0.023). TP53 mutations were identified in 24.2% of WNT and 29.4% of SHH tumors and were associated with inferior 3-year EFS (p<0.020). The proposed molecular stratification algorithm identified two prognostic groups with significantly different survival outcomes (p<0.001): the 3-year EFS was 62% in the unfavorable group and 87% in the favorable group.

Conclusion: These findings demonstrate substantial biological heterogeneity within standard-risk medulloblastoma. The proposed molecular stratification algorithm enables identification of prognostically unfavorable disease variants and may provide a practical framework for future risk-adapted therapeutic strategies.

Abstract 229

A "Drop" Too Many: The Cascading Crisis of Disseminated Pediatric Medulloblastoma

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Purpose: Medulloblastoma is the most common malignant brain tumor of childhood accounting for approximately 10% of all primary central nervous system (CNS) tumors. While dissemination through cerebrospinal fluid (CSF) pathway is recognized, simultaneous intracranial, intramedullary, and extramedullary spinal metastases at initial presentation are exceedingly rare and often mimics infectious or inflammatory conditions. We report an extensively disseminated medulloblastoma with widespread neuraxis involvement at diagnosis.

Methods: A five-year-old girl presented with progressive esotropia, headaches, and vomiting. Initial cranial imaging suggested a possible tuberculous granuloma and initially started on anti-Koch's therapy. Neurological progression prompted repeat MRI revealing diffuse intracranial disease with multiple lesions across the cerebrum, cerebellum, and brainstem and with intramedullary cervical (C1–C5) and extramedullary cauda equina (L3 L5) metastases. MRI spectroscopy showed an elevated choline-to-creatine ratio with reduced N-acetylaspartate, suggesting non-glial neoplasm. Due to the multifocal nature of the lesions, the tumors were deemed unresectable. Initial attempt of gross tumor resection with diagnostic biopsy and molecular profiling confirmed a non-WNT/non-SHH classic medulloblastoma, WHO Grade 4 with time to diagnose of approximately 1 year since initial presentation.

Results: Management included ventriculoperitoneal shunt placement and partial tumor debulking, followed by initial induction chemotherapy with consideration of primary CNS lymphoma. Treatment was transitioned to cisplatin-based protocol, incorporating craniospinal radiotherapy (36 Gy) and a posterior fossa boost (18 Gy) with weekly vincristine for medulloblastoma. Despite initial clinical improvement following multimodal therapy, the patient ultimately succumbed to disease progression with multiple episodes of seizure and deteriorating neurologic status.

Conclusion: This case highlights an aggressive presentation where extensive dissemination and multifocal involvement rendered the tumor unresectable at diagnosis and led to diagnostic delays. High clinical suspicion, early comprehensive neuroimaging and timely molecular profiling to differentiate pediatric malignancies from infectious etiology is warranted.

Abstract 230

High-Grade Thalamic Gliomas in Children: Treatment Challenges and Prognostic Factors

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DTC IIBS named after S. Berezin

Purpose: High-grade thalamic gliomas in children represent one of the most complex and understudied oncological pathologies in pediatric practice. Due to their rarity, they complicate diagnosis, optimal treatment selection, and outcome prediction. This abstract presents an analysis of the treatment of patients with this disease, allowing for the evaluation of the effectiveness of modern approaches and the identification of key factors affecting prognosis.

Methods: The study included 13 patients from the Treatment and Diagnostic Center of the Sergei Berezin International Institute of Biological Systems, who received complex therapy from 2018 to 2025. Information was collected through a retrospective analysis of medical records, by studying medical documentation requested from the patients' places of residence, as well as through direct examinations at our center

Results: The average age was 12 years (range from 7 to 17 years). Gender distribution: 7 males, 4 females. The K27M mutation was detected in 9 patients. Primary treatment included surgery, proton radiation therapy with concurrent chemotherapy. In 2 cases, craniospinal irradiation was initially required. Median overall survival (OS) was 12 months. Median progression-free survival (PFS) was

7 months. 4 patients (30%) are alive. 9 patients (70%) have died.

Conclusion: High-grade thalamic gliomas in children remain a rare and complex pathology requiring an individualized approach in diagnosis and treatment. Complex therapy (surgery + proton radiation therapy + chemotherapy) demonstrated limited effectiveness. Despite aggressive treatment, the prognosis for high-grade thalamic gliomas in children remains poor. Further research should focus on finding new therapeutic strategies to improve outcomes.

Abstract 231

Proton therapy of intracranial and intracanal Ewing's sarcoma

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DTC IIBS named after S. Berezin

Purpose: Radiation therapy (RT) is a leading method of treatment for achieving local control in patients with Ewing sarcoma (EwS). Abstract presents result of treating children with EwS of intracranial and intracanal localization using proton therapy (PT) within a single center. An evaluation of the effectiveness of initial and recurrences irradiation using different volumes was conducted.

Methods: The study included 18 patients from DTC IIBS named after S. Berezin who received PT from 2018 to 2024, of whom 12 (66.6%) had intracranial localization and 6 (33.3%) had intracanal. Information was collected through retrospective analysis of medical records, by studying medical documentation requested from the patients' places of residence, as well as during examinations of patients directly at our center.

Results: The median age at diagnosis was 9 years (2–16 years) for patients with intracranial localization and 8 years (2–16 years) for those with intracanal. The median event-free survival for intracranial cases was 20 months, and overall survival was 39.5mth; for intracanal it was 30mth and 31mth, respectively. PT at disease debut was performed in 15 patients (OS 41mth, EFS 22.5mth). Local irradiation at debut was received by 7 patients with intracranial localization (OS 41mth) and 5 with intracanal (OS 32mth). Craniospinal irradiation (CSI) at disease debut was received by 2 patients with intracranial localization (OS 42.5mth) and 1 with intracanal (OS 30mth). Three patients did not receive RTduring primary treatment (OS 37 months, EFS 14 months). For recurrence treatment among intracranial cases, local irradiation was received by 3 patients (OS 39.5mth), and CSI by 2 (OS 40,5mth).

Conclusion: RT provides good local control in primary treatment and recurrences of EwS. The use of CSI appears to be a promising method for preventing tumor spread. Consideration should be given to a personalized approach in determining irradiation volumes for this group of patients.

Abstract 232

Primary Spinal Cord Tumors: A Single-Center Experience

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Purpose: Primary spinal cord tumors are extremely rare, accounting for only 5-10% of all CNS tumors. Due to their rarity, published data is limited to small series and case reports. The aim is to present a single-center experience in treating pediatric primary spinal cord tumors.

Methods: A retrospective analysis of 25 patients was conducted. The cohort included: astrocytomas (n=2, 8%; 1 anaplastic, 1 pilocytic), ATRT (n=2, 8%), gliomas (n=6, 24%), and ependymomas (n=15, 11 spinal (73%), 4 myxopapillary (27%)).

Results: Median age at diagnosis varied by histology: spinal ependymomas (9.5 years), myxopapillary ependymomas (8 years), gliomas (7.6 years), ATRT (5 and 6 years), and astrocytomas (1 and 4 years). Treatment approaches differed across groups. Among spinal ependymomas, 8 patients (72.7%) underwent subtotal tumor resection (STR) and 3 (27.3%) gross total resection (GTR). All received proton therapy (36% craniospinal, 64% local); 2 received second-line chemotherapy, 1 had re-irradiation. All myxopapillary ependymomas had STR and local radiotherapy (RT). All glioma patients achieved STR, local RT and adjuvant chemotherapy. Both ATRT patients had STR, neoadjuvant/adjuvant chemotherapy and radiotherapy (1 craniospinal, 1 local). Both astrocytoma patients achieved STR with radiotherapy (1 craniospinal, 1 local); 1 received adjuvant chemotherapy. Survival outcomes demonstrated significant histology-dependent variation. Astrocytomas showed 5-year overall survival (OS) and event-free survival (EFS) of 100%. ATRT patients had 2-year OS of 100% but EFS of 50%. Gliomas demonstrated poorest outcomes with 2-year OS of 50% and EFS of 17%. Spinal ependymomas showed 2-year OS of 100% and EFS of 66%, while myxopapillary ependymomas achieved 3-year OS and EFS of 100%.

Conclusion: Primary spinal cord tumors remain exceptionally rare. Survival outcomes varied significantly depending on the histological tumor type. Patients with ependymomas and astrocytomas demonstrate superior survival compared to those with gliomas and ATRT. Larger multicenter studies are needed for comprehensive multifactorial analysis.

Abstract 233

Nanoparticles with iRGD-DSPE-PEG for CNS drug delivery and anti-glioma therapy

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Purpose: This study aimed to construct an arsenic trioxide (ATO)-loaded nanodrug delivery system, termed iRGD-PEG-ATO, using DSPE as the carrier modified with polyethylene glycol (PEG) and conjugated with the tumor-targeting peptide iRGD. The system was designed to overcome the limitations of free ATO, including poor blood-brain barrier (BBB) penetration, rapid systemic clearance, narrow therapeutic window, and lack of tumor-specific distribution, thereby providing a novel strategy for glioma therapy.

Methods: The targeted nanocarrier iRGD-PEG-ATO was synthesized based on iRGD-DSPE-PEG and the αvβ3 integrin-targeting ligand. The nanoparticles were characterized using XPS, TEM,FTIR. In vitro drug release was evaluated via the dialysis bag method. Cytotoxicity and antitumor efficacy were assessed using MTT and live/dead assays. Flow cytometry was employed to analyze effects on cell apoptosis and cell cycle. An in vitro BBB model co-cultured with U87 glioma cells was established to study the tumor-suppressive effects of different ATO formulations after crossing the BBB. An orthotopic U87-LUC glioma model in nude mice was constructed to evaluate the tumor inhibition and survival benefits of iRGD-PEG-ATO. Ki67 and TUNEL immunohistochemistry were performed to assess its effects on tumor cell proliferation and apoptosis in vivo. Transcriptome sequencing was conducted on cells treated with the nanomaterial. Western blot and IHC were used to examine TGFBR2 protein expression in glioma cells and to investigate the impact on the MAPK pathway following treatment with iRGD-PEG-ATO alone or in combination with temozolomide.

Results: In vitro release studies showed that iRGD-PEG-ATO released more ATO at pH 5.6 (42.7% within 24 hours) compared to pH 7.4. In the in vitro BBB model, iRGD-PEG modification enhanced the cytotoxicity of the DSPE-loaded ATO, attributable to increased uptake by U87 cells. Live/dead and apoptosis assays demonstrated that iRGD-PEG-ATO increased apoptosis by approximately 60% compared to free ATO. Furthermore, iRGD-PEG-ATO extended the plasma half-life by about 1-fold and significantly improved antitumor efficacy compared to free ATO. Treatment with iRGD-PEG-ATO prolonged the survival of tumor-bearing mice by approximately 2-fold relative to free ATO. Transcriptome sequencing indicated downregulation of the TGFBR2 gene in the nanomaterial-treated group, suggesting involvement of the MAPK pathway. Additionally, temozolomide was found to enhance the efficacy of the nanomaterial, which was corroborated by WB and IHC analyses.

Conclusion: The developed iRGD-PEG-ATO nanocarrier effectively crosses the BBB, accumulates within glioma cells, and releases its drug payload. This study demonstrates that iRGD-PEG-ATO enhances the antitumor efficacy of ATO against glioma, offering a promising targeted therapeutic strategy.

Abstract 234

Clinical Analysis and Literature Review of Fanconi Anemia Complicated with Medulloblastoma(Report of 2 Cases)

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Purpose: Fanconi anemia is a rare disease with low incidence, and its concurrent occurrence with medulloblastoma is even rarer.

Methods: The clinical characteristics, laboratory examinations, treatment courses and complications of 2 patients with Fanconi anemia type D1 complicated with medulloblastoma in our hospital were retrospectively analyzed, combined with a review and discussion of relevant domestic and foreign literature.

Results: Among the 2 FA patients, Case 1 was male and Case 2 was female. Case 1 was diagnosed with FA at 5 months of age, while Case 2 was diagnosed with FA at 4 years and 6 months (1 year and 4 months after medulloblastoma treatment). Case 1 developed medulloblastoma at 2 years and 9 months, located in the posterior fossa; Case 2 developed medulloblastoma at 3 years and 2 months, involving supratentorial and infratentorial regions of the posterior fossa. The main clinical manifestations of Case 1 were headache and unsteady gait; Case 2 presented with unsteady gait only. Neither patient had a family history of hematological diseases, and their parents were non-consanguineous. Both patients had café-au-lait spots, pigmentation, depigmentation, finger deformities, microcephaly and mental retardation. Case 1 additionally had hypospadias. Pathological examination after intracranial tumor resection confirmed medulloblastoma in both cases: Case 1 was SHH subtype, desmoplastic/nodular type with TP53 mutation, WHO grade ; Case 2 was SHH subtype with wild-type TP53, WHO grade . Case 1 received 1 cycle of chemotherapy after tumor resection, followed by bone marrow failure and fungal sepsis, and eventually died after treatment abandonment. Case 2 received 5 cycles of chemotherapy after tumor resection, with repeated severe myelosuppression and admission to ICU due to severe infection. Subsequently, 6 sessions of craniospinal irradiation were administered but discontinued due to myelosuppression. Tumor recurrence occurred, and a second tumor resection was performed. Four months after the operation, the tumor relapsed again, and local head and neck radiotherapy was given. Spinal dissemination metastasis occurred 1 month after radiotherapy completion, and the patient died after treatment abandonment.

Conclusion: For tumor patients accompanied by physical deformities, etiological screening should be emphasized to rule out Fanconi anemia. For Fanconi anemia with BRCA2 genotype complicated with medulloblastoma, chemotherapy dose reduction is recommended to lower the incidence of severe myelosuppression. Allogeneic hematopoietic stem cell transplantation should be performed when the tumor achieves complete remission. The prognosis of Fanconi anemia complicated with medulloblastoma is poor.

Abstract 235

The impact of residual tumor assessed at the end of first-line treatment on the risk of recurrence in patients with medulloblastoma

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Purpose: The aim of this study is to investigate the impact of residual tumor on the risk of medulloblastoma relapse, which is due to the ambiguity of existing data and the relevance of the problem of disease recurrence.

Methods: The study included 66 patients with newly diagnosed medulloblastoma with residual tumor after surgery (R+M0, R0M1–4, R+M1–4, Chang staging) treated according to HIT-MED protocols v4.0 (2017) and v5.1 (2020) during 2017–2023. Median age at diagnosis was 8.6 years (range 1.5–17.9). Initial staging: R+M0 – 18 (27.3%), R0M1–4 – 10 (15.2%), R+M1–4 – 38 (57.5%)

Results: Eight-year OS and EFS for the cohort (n=66) were 70.7% ± 6.7% and 42.7% ± 7.2%. Response to first-line treatment was evaluated in 61 (92.4%) patients. At treatment completion, MRI confirmed complete response (R0M0) in 16 (26.2%), while 45 (73.8%) had residual disease (R+M0, R+M1–4, R0M1–4). Eight-year CIR was 25.0% ± 11.2% in patients with complete response and 57.2% ± 8.2% with residual tumor (p=0.1003). Among patients with residual disease, eight-year EFS was 75.0% ± 11.0% for R+M0, 20.4% ± 9.5% for R+M1–4, and 33.3% ± 19.2% for R0M1–4 (p=0.0011). Patients without metastases at therapy completion (n=34) had significantly better outcomes than those with metastatic disease (n=27): eight-year EFS 67.3% ± 10% vs 23.8% ± 8.6% (p=0.0004). Corresponding CIR values were 25.2% ± 8% and 76.2% ± 9.1% (p=0.0002).

Conclusion: Residual tumor at treatment completion is associated with an approximately twofold higher relapse risk compared with complete response. The poorest EFS occurred in patients with R+M1–4 disease. Persistent metastatic disease after therapy completion is associated with a increased relapse risk.

Abstract 236

Pragmatic Molecular Risk Stratification of Pediatric Medulloblastoma in a Resource-Limited Setting: A Cohort Study from Kazakhstan

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Purpose: Medulloblastoma is the most common malignant brain tumor in children and is characterized by significant molecular heterogeneity. Current classification recognizes four molecular subgroups (WNT, SHH, Group 3, and Group 4), which differ in biology, prognosis, and require risk-adapted treatment strategies. In high-income countries, molecular stratification based on DNA methylation profiling and next-generation sequencing has become a key component of modern diagnostics. However, access to these technologies remains limited in many parts of Central Asia, making implementation of contemporary molecular classification challenging in routine clinical practice. The aim of this study was to develop and evaluate a resource-adapted diagnostic algorithm for molecular stratification of pediatric medulloblastoma.

Methods: We conducted a retrospective study of pediatric patients (0–18 years) with histologically confirmed medulloblastoma treated at the Corporate Fund “University Medical Center” in Astana, Kazakhstan, between 2015 and 2023. A total of 68 patients were included based on the availability of archival tumor tissue and clinical follow-up data. A surrogate diagnostic algorithm was developed using an integrated approach that combined histopathological evaluation, immunohistochemical markers, and assessment of TP53 status. The resulting classifications were analyzed in relation to clinicopathological characteristics and survival outcomes.

Results: The proposed algorithm enabled stratification of patients into molecularly associated groups: WNT, SHH-TP53 wild-type, SHH-TP53 mutant, Group 3, and Group 4. Clear differences in clinical course and treatment outcomes were observed among these groups. The most favorable outcomes were seen in patients with WNT and SHH-TP53 wild-type tumors, whereas the poorest outcomes were observed in patients with SHH-TP53 mutant and Group 3 tumors. Overall, the survival patterns in our cohort were consistent with prognostic trends reported in international medulloblastoma studies.

Conclusion: The proposed resource-adapted algorithm allows clinically meaningful molecularly informed risk stratification of pediatric medulloblastoma in settings where access to comprehensive molecular testing is limited. This approach may be useful in routine diagnostic practice and could support the implementation of risk-adapted treatment strategies in resource-constrained healthcare systems. Acknowledgment: The authors gratefully acknowledge the support and collaboration of Nagasaki University, particularly Professor Masahiro Nakashima.

Abstract 237

SUBCUTANEOUS MYXOPAPILLARY EPENDYMOMA IN CHILDREN: A SINGLE CENTER EXPERIENCE

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Purpose: Subcutaneous myxopapillary ependymoma (MPE) in children is an exceptionally rare entity, most often arising in the sacrococcygeal region and potentially being misdiagnosed as benign soft tissue lesions.

Methods: A retrospective analysis of 5 pediatric patients with histologically confirmed subcutaneous MPE was performed. Clinical presentation, imaging, pathology, treatment details and outcomes were extracted from medical records.

Event-free survival (EFS) was calculated from the date of diagnosis to progression or last follow-up.

Results: Five female patients with subcutaneous MPE were identified. Median age at diagnosis was 8.3 years (range 4.9–15.3 years). Four tumors were located in the sacrococcygeal subcutaneous tissue, while one was presacral with pelvic extension. The most common presentation was a painless subcutaneous mass in the sacrococcygeal or intergluteal region; the presacral tumor caused constipation and abdominal pain due to rectal compression. All patients underwent surgery as initial treatment: gross total resection (R0) in 4 cases and subtotal resection (R+) in 1 case. No metastases were recorded at diagnosis. Histology confirmed WHO grade 2 MPE in all patients, with mitotic activity ranging from absent to moderately increased and a Ki-67 index generally ≤10%. The R+ patient received multi-agent chemotherapy (cyclophosphamide followed by etoposide/cisplatin/ifosfamide) and additional systemic therapy (topotecan/carboplatin) at local progression, whereas R0 patients had surgery alone. With a median follow-up of 42.9 months (range 13.4–120.5 months), one local progression occurred 4.8 months after diagnosis; all patients are alive, and 4 remain progression-free.

Conclusion: Pediatric subcutaneous MPE most frequently presents as an isolated, painless sacrococcygeal mass and can be under-recognized. Gross total resection alone appears sufficient for durable local control in most patients, while incomplete resection may be associated with early local progression despite adjuvant systemic therapy.

Abstract 238

Optimizing Diagnosis and Treatment in Pediatric Supratentorial Ependymoma: Results Retrospective Molecular Analysis

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Purpose: WHO 2021 mandates molecular diagnosis for pediatric STE, but many centers rely on histology alone, causing misclassification. Complex methods delay RT, harming survival. We analyzed patients initially diagnosed with STE to assess misdiagnosis rate and outcomes in confirmed cases

Methods: Retrospective molecular analysis (dPCR, NanoString, methylation) was performed on archival tumor from 69 children initially diagnosed with STE (2012–2024). All underwent surgery; confirmed STE received photon/proton therapy. Survival (EFS, OS) was analyzed.

Results: STE confirmed in 60/69 (87%); 13% reclassified as other entities. GTR 68%; median RT 33 days. 5-year EFS 68.4% (vs ACNS0831 69.6%), OS 86.9%. RT delay >40 days (p=0.03) and incomplete resection (p=0.02) worsened EFS; other factors insignificant

Conclusion: Retrospective analysis revealed 13% histological misdiagnosis, leading to suboptimal treatment. Fast-track PCR confirms ZFTA/YAP1 fusions in most STEs within days, enabling timely RT (<40 days). Extended testing for PCR-negative cases essential. 5-year EFS (68.4%) and OS (86.9%) align with international benchmarks. Prospective validation warranted.

Abstract 239

Improving Outcomes in Pediatric Supratentorial Ependymoma: The Critical Role of Molecular Confirmation and Timely Radiotherapy

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Purpose: WHO 2021 mandates molecular diagnosis for pediatric STE, but many centers rely on histology alone, causing misclassification. Complex methods delay RT, harming survival. We analyzed patients initially diagnosed with STE to assess misdiagnosis rate and outcomes in confirmed cases

Methods: Retrospective molecular analysis (dPCR, NanoString, methylation) was performed on archival tumor from 69 children initially diagnosed with STE (2012–2024). All underwent surgery; confirmed STE received photon/proton therapy. Survival (EFS, OS) was analyzed.

Results: STE confirmed in 60/69 (87%); 13% reclassified. GTR in 68%; median RT 33 days. 5-year EFS 68.4%, OS 86.9%—matching ACNS0831 (69.6%). RT delay >40 days (p=0.03) and incomplete resection (p=0.02) worsened EFS; other factors showed no impact

Conclusion: Retrospective analysis revealed 13% histological misdiagnosis, leading to suboptimal treatment. A fast-track PCR algorithm confirms ZFTA/YAP1 fusions in most true STEs within days, enabling timely RT (<40 days). Extended testing remains essential for PCR-negative cases. 5-year EFS (68.4%) and OS (86.9%) align with international benchmarks, underscoring our approach's effectiveness. Prospective validation warranted.

Abstract 240

Molecular pathological diagnostic issue of pediatric midline glioma in Mongolia

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Purpose: H3 K27M-mutant diffuse midline glioma is associated with poor prognosis. We emphasize that diffuse gliomas centered in midline structures harboring H3 K27M mutation fulfill the diagnostic criteria for the entity “diffuse midline glioma, H3 K27M mutant”.

Methods: The study was carried out from January 2015 to January 2026 in the National Center for Pathology, Ulaanbaatar, Mongolia. A total of 67 cases were enrolled. Immunohistochemical staining for histone H3-K27M mutant protein was performed using formalin-fixed paraffin-embedded TMA blocks.

Results: In this study, out of 67 cases, 34 were midline glioma. H3K27M immunohistochemistry expression was found in 8/34 samples. According to the 5th WHO classification of tumors of CNS, 8 (23.5%) cases out of 34 were diagnosed as H3 K27M-mutant diffuse midline glioma.

Conclusion: H3K27M status is a very important supplement to histological grading of gliomas for appropriate clinico-pathological diagnosis. Testing for H3K27M mutations should be pursued in all pediatric midline gliomas independent of their histologic appearance or grade. In our study, H3K27M mutated pediatric midline glioma is highest prevalence in the female, 5-9 age group.

Abstract 241

Impact of molecular group on survival outcomes in patients with ATRT CNS

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Purpose: ATRT CNS is an aggressive tumor with a poor prognosis. Molecular group testing has an impact on prognosis in patients with ATRT CNS.

Methods: The analysis included 57 patients who underwent molecular biology testing of their tumors. The patients were classified according to molecular group: TYR 22, SHH 26, MYC 9. TYR was more common among girls, MYC among boys, and SHH was found in equal proportions in both sexes. In children under 12 months, the TYR group was more common (40.9%), while in patients over 12 months, the MYC (88.9%) and SHH (76.9%) groups were more common. In the TYR molecular group, tumors were more often localized infratentorial (72.7%), in the MYC group – supratentorial (66.7%), and in the SHH group, infra- and supratentorial tumors were encountered in equal proportions. Metastatic lesions were most frequently detected in the SHH group (38.5%), and less frequently in the TYR (13.6%) and MYC (11.1%) groups. Total and subtotal tumor resection was performed in 95.5% of patients with the TYR molecular group, 69.2% with SHH, and 33.3% with MYC. 34 patients received treatment according to the ATRT 2006 protocol, the remaining patients received treatment according to various protocols. Thirty-five patients received RT (8 with local irradiation, 27 with local irradiation). Regional chemotherapy was performed in 45 patients, and HDCT was administered to 9 patients.

Results: Disease recurrences were detected within 22–29 months from diagnosis; all these patients subsequently died: TYR molecular group – 5, MYC – 9, SHH – 21. Children with the TYR molecular group had a statistically significantly better

PFS compared to those with SHH and MYC molecular groups: 0.76, 0.15, and 0.0, respectively, p <0.001. A higher OS rate was observed in children with the TYR molecular group compared to those with SHH and MYC molecular groups: 0.76, 0.29, and 0.0, respectively, p = 0.0061. In a multivariate analysis, regional chemotherapy, molecular group, and RT influenced PFS. OS was influenced by RT, HDCT, and molecular group.

Conclusion: Molecular group influenced treatment outcomes. Patients with the TYR molecular group had the highest PFS and OS rates, while those with the MYC molecular group had the worst outcomes. Survival was also influenced by the presence of regional chemotherapy, RT, and HDCT.

Abstract 242

Shunt-Associated Intra-Abdominal Metastasis of Medulloblastoma in a Child: A Case Report

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Purpose: To present a clinical case of a patient who received antitumor treatment at the Research Institute of Pediatric Oncology and Hematology.

Methods: Patient K., a 12-year-old, diagnosed with: "Medulloblastoma of the cerebellar vermis and left cerebellar hemisphere. Classic histological variant. Surgical treatment on 03/31/2023. R+ M3- status in the postoperative period. Programmed treatment according to the HIT MED 2020 protocol."

Results: Six months after the end of therapy, no signs of progression of the primary disease within the central nervous system were detected; however, the appearance of a space-occupying mass in the pelvis was noted. Histological examination results: Medulloblastoma.

Conclusion: Shunt-associated metastasis is a complex and poorly understood topic in pediatric oncology. To date, the issue of preventing shunt-associated metastasis remains debatable

Abstract 243

Malignant Gliomas in Children: A Single-Center Study Including 103 Cases

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Purpose: High-grade gliomas (HGG) are a heterogeneous group of primary central nervous system tumors in children with an extremely poor prognosis. The aim of this study is to retrospectively analyze 103 pediatric patients diagnosed with malignant gliomas and treated at our center. The primary objectives are: (1) to determine the demographic characteristics and histologic spectrum of HGGs in this cohort; (2) to determine clinical outcomes by assessing the timing of disease progression and overall survival

Methods: This was a retrospective, single-center cohort study conducted at the Moscow Institute of Cardiology and Imaging (MIBS). We reviewed the medical records of all pediatric patients (aged 0–18 years) diagnosed with HGG between January 2018 and December 2025. A total of 103 cases were identified. Inclusion criteria were: (1) histopathological diagnosis of high-grade glioma (WHO grade III or IV); (2) age at diagnosis ≤18 years; and (3) availability of complete medical records and follow-up data. Patients with low-grade gliomas were excluded.

Results: The median age of patients was 10 years (range: 2-18 years). With a median follow-up of 30 months (range: 4-170 months), the median overall survival (OS) for the entire cohort was 19.5 months. The median progression-free survival (PFS) was 12.6 months. In univariate analysis, resection stage was a significant predictor of improved OS (Current resection was significantly associated with improved median OS – not reached vs. 18 months, p<0.0001). Metastatic CNS involvement was associated with a reduced overall survival rate – 2.8 years versus 1.3 years (p<0.05). Furthermore, for certain groups of malignant glial tumors occurring in children, such as pleomorphic xanthoastrocytoma (Gr III) and astroblastoma (Gr III) (a group of circumscribed tumors), the 5-year overall survival rates were 78% and 75%, respectively. The 5-year event-free survival rates for the same tumors were 66% and 67%, respectively

Conclusion: Despite modern surgical and therapeutic options (chemotherapy, radiation therapy, molecularly targeted therapy, etc.), the prognosis for these tumors remains disappointing, which provides an opportunity for further research and identification of the most promising avenues for the management of these patients

Abstract 244

Outcomes of Treatment of Children Younger Than 5 Years of Age with SHH Medulloblastoma

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Purpose: Medulloblastoma of the SHH molecular group (MB-SHH) is characterized by a different prognosis depending on the age and molecular characteristics of the tumor. The factors influencing the outcome of the disease are still being discussed.

Methods: A retrospective analysis of 61 patients with MB-SHH under the age of 5 years treated in the period from 1994 to 2021 was performed. The median age at diagnosis was 2 years (0.5–4 years). All histological variants were encountered: desmoplastic, classical, large-celled, with nodular extensiveness. Metastatic lesions (M1-M3) were detected in 29.5% of patients, residual tumors of more than 1.5 cm3 were detected in 39.3%. Germinal mutation of TP53 was found in 6.6% of cases, amplification of the NMYC gene in 9.8% of cases. Patients were divided into risk groups: low – 15%, standard – 13%, high – 66%, very high – 6%.

Results: at the time of analysis, 76.7% of patients were alive, 23.3% died. Relapse of the disease was registered in 41% of patients. 10-year progression-free survival (PFS) was 62.1%, overall survival (OS) was 65.9%; The median overall survival was 93 months. Gender, presence of residual tumor, and stage of disease had no statistically significant effect on survival. At the same time, the risk groups significantly differed in survival rates (p<0.01): 10-year OS was 100% in the standard risk group, 88.9% in the low-risk group, 71.6% in high-risk, and 25% in the very high-risk group. Similar differences were noted for PFS. The best survival was in desmoplastic and medulloblastoma with extensive nodular (p<0.01). The presence of a germinal mutation in the TP53 gene and MYCN amplification were associated with a significant deterioration in survival (p<0.001). Secondary tumors were registered in 4 patients.

Conclusion: The results of treatment were influenced by mutations in the TP53 and MYCN genes, as well as the histological variant. The presence of residual tumor, disease stage, and mutations in the genes of the SHH pathway did not have a statistically significant effect on disease outcome.

Abstract 245

Efficacy and Safety of Trametinib Use in Pediatric Brain Tumors in a Low to Middle Income Country.

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Purpose: Trametinib, a mitogen activated protein kinase (MEK) inhibitor, is a promising targeted therapy for children with progressive, unresectable low-grade gliomas (LGGs) who have failed standard chemotherapy regimens. There are limited data on the use of targeted therapy in Low-Middle-Income Countries (LMIC), and specifically, there are no published data from Lebanon on the use, efficacy, and safety of these agents.

Methods: Following IRB approval, a retrospective review of the medical records of pediatric patients with brain tumors who received trametinib at the Lebanese American University Medical Center Rizk hospital was undertaken, along with a detailed data collection and a family interview to evaluate the quality of life of the patients while administering this agent.

Results: Four pediatric patients diagnosed with progressive LGGs, harboring distinct molecular drivers (KIAA1549-BRAF, NF1 Loss, and BRAF V600E), were identified. Age range at diagnosis was 11 months to 10 years. Trametinib was obtained on a compassionate basis through an international Managed Access Program. The median duration of trametinib use was 30.2 months (range 22 to 35.5 months). Clinical improvement was observed in all patients along with partial

radiographic response in one patient. The most common side effects were skin reactions and gastrointestinal complaints, with few therapy interruptions required. Quality-of-life assessment using the Pediatric Quality of Life Inventory™ 4.0 Generic Core Scales revealed significant heterogeneity, with physical and school function being the most affected.

Conclusion: Despite few manageable side effects, the favorable outcomes on tumor response and quality of life of trametinib-based therapy in Lebanese pediatric LGG patients demonstrate that the use of MEK inhibitors should be considered in LMIC settings.

Abstract 246

Targeted Therapy in Pediatric Low Grade Glioma: A Systematic Review of Clinical Outcomes in the Molecular Era

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Purpose: Pediatric low-grade glioma (pLGG) is the most common childhood brain tumor, driven mainly by MAPK pathway alterations such as BRAF mutations and fusions. Conventional chemotherapy is effective in many patients but has limited efficacy and cumulative toxicity in progressive or unresectable disease.

Methods: To systematically review the clinical evidence supporting MAPK-pathway–targeted therapies in pLGG

Results: Phase II trials showed selumetinib activity in recurrent/progressive pLGG (PR 42%, ~78% 2-year PFS). Dabrafenib plus trametinib was superior to carboplatin/vincristine (ORR 47% vs 11%). In FIREFLY-1, tovorafenib achieved durable responses in ~50% of patients.

Conclusion: Targeted inhibition of the MAPK pathway now represents a cornerstone of precision therapy in pediatric low-grade glioma and should be considered standard of care in appropriately selected patients.

06. SOLID TUMORS — ORAL PRESENTATION

Abstract 247

Outcomes of GD2-positive refractory/relapsing forms of bone and soft tissue sarcomas chemoimmunotherapy in children

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Purpose: Immuno-oncological drugs have been increasing over the past decade. The purpose of the study was to evaluate the outcomes of treatment for GD2-positive sarcomas in children who were given antiGD2 monoclonal antibodies (MA).

Methods: 52 children were included in the study who had refractory/recurrent bone and soft tissue sarcomas. In 22 (42%) children, soft tissue sarcoma (STS) was detected, in 19 (37%) - osteosarcoma (OS), and in 11 (21%) - Ewing sarcoma (ES). The level of GD2 expression in tumor samples ranged from 1.1% to 95%, with a mean of 39±5%. All patients with STS and OS successfully underwent local treatment (radiotherapy and/or surgery), local treatment for Ewing sarcoma was limited to 36% of cases.

Results: Overall objective response was 56% (29/52): 26 patients (50%) achieved complete remission (CR) and 3 (6%) achieved partial remission (PR). The disease was controlled 65% (34/52) (CR + PR + stabilization). The disease progression was diagnosed in one-third (17/52) of patients. One-year OS was 81 ± 6%, PFS - 56 ± 8%. There were no significant differences in survival rates (p>0.005) after analyzing long-term outcomes. Patients with STS had a Me PFS of 25 months, but OS patients did not achieve it, and ES patients had a Me PFS of 12 months. Higher rates of PFS were in children with OS (67±12%), followed by patients with STS (53±13%), and the worst results were in the cohort with ES (22±19%).

Conclusion: In cases of refractory/relapsing sarcomas, the use of antiGD2 therapy against tumors expressing the GD2 antigen is encouraging. This approach can manage the malignant process in almost two-thirds of patients (65% of cases), which leads to a significant increase in the period without disease progression (up to 56%).

Abstract 248

Clinical Characteristics and Prognosis Analysis of Extracranial Malignant Rhabdoid Tumor in Children

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Purpose: To investigate the clinical characteristics and prognosis of extracranial malignant rhabdoid tumor (eMRT) in children, and to provide a reference for the clinical treatment of this disease.

Methods: A retrospective analysis was conducted on the clinical data of newly diagnosed eMRT children treated in the Department of Oncology, Beijing Children's Hospital, Capital Medical University from March 2009 to December 2024. Their clinical characteristics were summarized, and survival analysis as well as prognostic risk factor analysis were performed.

Results: A total of 43 children with eMRT were enrolled in this study, the median age at diagnosis was 20 months (range: 2–138 months) in the entire cohort. Among them, 24 cases were malignant renal rhabdoid tumors and 19 cases were extracranial, extrarenal rhabdoid tumors. Of the 43 children, 23 cases (53.5%) were complicated with distant metastasis. Twenty-nine (67.4%) underwent primary tumor resection. Among the children, 24 (55.8%) underwent gross total resection (GTR), 5 (11.6%) partial resection, and 14 (32.6%) biopsy only. Their 3-year overall survival (OS) rates were 40.8%, 35.3%, and 33.3%, respectively (P=0.711). Chemotherapy was administered to 42 children (97.7%); among those who were ineligible for GTR and had poor chemotherapy sensitivity, the 3-year OS rate was significantly lower than that of chemotherapy-sensitive children (28.6% vs. 58.2%, P=0.041). Radiotherapy was given to 15 children (34.9 %), with the 3-year OS rate in the radiotherapy group being higher than that in the non-radiotherapy group (69.1% vs. 32.9%, P=0.026). The 3-year event-free survival (EFS) and OS rates of all 43 children were 31.4% and 44.3%, respectively. Multivariate Cox regression analysis showed that a diagnostic age < 12 months was significantly associated with a higher risk of death (hazard ratio [HR] = 3.7, 95% confidence interval [CI]: 1.2–11.1, P=0.02).

Conclusion: Children with eMRT have an overall poor prognosis. A diagnostic age <12 months is an independent risk factor for higher mortality in these children. Further large-scale, long-term follow-up studies are needed to explore the prognostic factors of this disease.

Abstract 249

Treatment outcomes of stage III - IVa nasopharyngeal carcinoma in children at Vietnam National Cancer Hospital

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Purpose: Nasopharyngeal carcinoma (NPC) is a rare malignancy in children, accounting for approximately 1% of pediatric cancers. It is most commonly diagnosed during adolescence and often presents with advanced locoregional disease. Since January 2019, pediatric patients (<18 years) with stage III–IVA NPC at Vietnam National Cancer Hospital have been treated according to the SIOP protocol, with encouraging responses observed. This study aimed to (1) describe the clinical and paraclinical characteristics of pediatric stage III–IVA NPC at K Hospital and (2) evaluate treatment outcomes.

Methods: A retrospective–prospective descriptive study was conducted on 38 patients aged <18 years with stage III–IVA NPC treated according to the SIOP protocol.

Results: The mean age was 13.39 ± 2.75 years (range 7–17), with a male-to-female ratio of 2.8. The most common presenting symptom was cervical lymphadenopathy (78.9%). All patients had WHO type III histology. Plasma EBV-DNA ≥300 copies/ml was detected in 22/27 tested patients. Disease stages were III (57.9%) and IVA (42.1%). After induction chemotherapy, partial response was observed in 94.7% of patients, stable disease in 2.6%, and progressive disease in 2.6%. At the end of treatment, complete response was achieved in 50% and partial response in 47.4%. After a median follow-up of 27 months, the 3-year overall survival (OS) and progression-free survival (PFS) were 100% and 84.8%, respectively. Factors potentially associated with poorer PFS included age ≥10 years, pretreatment EBV-DNA ≥300 copies/ml, and stage IVA, but these differences were not statistically significant (p>0.05). Grade 3–4 neutropenia occurred in 21.1% during induction chemotherapy and 21.0% during concurrent chemoradiotherapy. Common non-hematologic toxicities were nausea/vomiting (76.3%), dermatitis (84.2%), and oral mucositis (94.7%). Frequent late toxicities included skin fibrosis (39.5%), xerostomia (39.5%), and trismus (10.6%).

Conclusion: Pediatric NPC shows favorable responses to combined chemotherapy and radiotherapy, with excellent survival outcomes. However, careful monitoring of treatment-related toxicities, particularly late effects, remains essential.

Abstract 250

Neonatal Solid Tumors: A Single-Center Experience and Evaluation of Prognostic Factors

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Purpose: Neonatal solid tumors account for approximately 2% of childhood malignancies yet differ from tumors in older children regarding biological behavior and treatment response. Published 1-year overall survival (OS) rates range from 60-84%, with metastatic disease carrying poor prognosis. Data from Turkey remain limited. This study evaluated survival and prognostic factors separately for malignant and benign neonatal solid tumors.

Methods: Patients diagnosed with solid tumors in the neonatal period (0-28 days) between December 2019 and February 2026 at a single tertiary center were retrospectively reviewed. Survival was analyzed using Kaplan-Meier and log-rank methods. Prognostic factors were evaluated separately for malignant and benign tumors using Cox regression. Neuroblastoma, comprising over half of malignant cases, was analyzed separately.

Results: Fifty-three patients were included; 31 (58.5%) male, median gestational age 37 weeks (25-41), median diagnosis age 4 days. Preterm birth was noted in 39.6% and antenatal diagnosis in 62.3%. Most common diagnoses were rhabdomyoma (30.2%), neuroblastoma (26.4%), teratoma (18.9%), rhabdomyosarcoma and CNS tumors (5.7% each), with six other diagnoses at 1.9-3.8% each. Twenty-six (49.1%) had malignant and 27 (50.9%) benign tumors. After median follow-up of 7 months, 13 (24.5%) died. Twelve-month OS was 53.7% for malignant and 88.7% for benign cases (p=0.046). Among malignant patients, metastasis at diagnosis was the sole independent prognostic factor (HR=5.48; 95% CI: 1.33-22.58; p=0.019). Median OS for metastatic cases was only 3 months, while most non-metastatic patients remained alive with median not reached (p=0.008). In the neuroblastoma subgroup (n=14), metastatic median OS was 3 months while non-metastatic patients remained alive (p=0.001). All three benign deaths were due to prematurity complications rather than tumor progression.

Conclusion: Metastasis at diagnosis is the strongest independent prognostic factor in malignant neonatal tumors. Mortality in benign tumors is driven by prematurity complications rather than tumor itself, supporting individualization of management based on malignancy status.

Abstract 251

Laparoscopic Major Hepatectomy in Children: A Report of 31 Cases

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Purpose: To evaluate the safety, feasibility, and oncological outcomes of laparoscopic major hepatectomy pediatric liver tumors

Methods: Details of perioperative workup and management, and postoperative outcomes of pediatric patients with liver masses who underwent laparoscopic major hepatectomy from November 2005 to June 2024 were retrospectively analyzed. Relevant indicators were compared across different types of hepatectomy

Results: A total of 31 pediatric patients were included, comprising 16 males and 15 females, with a mean surgical age of 29.3 months (range: 5-113 months). Among them, 3 cases were benign, and 28 were hepatoblastoma (HB). The average maximum tumor diameter was 6.3 cm (range: 3.2-14.4cm). Anatomical resection, besides 1 case of ALPPS and 1 central hepatectomy, were performed, including Group A (left hemihepatectomy, n=17) and Group B (right hemihepatectomy, n=12). The mean operative time was 233.6±56.3 minutes for Group A, 295.7±51.2 minutes for Group B, with a statistically significant difference between Groups A and B (adjusted P=0.033). Intraoperative blood loss was 67.7±59.4 mL for Group A, 74.5±62.6 mL for Group B with no significant differences (F=0.12, P=0.889). Blood transfusions were required in 18 cases, and 4 cases were converted to open surgery. Postoperative bile leakage occurred in 3 cases, of which 2 resolved spontaneously after drainage and 1 required reoperation. All HB cases achieved R0 resection, with a mean margin of 1.0±0.3cm (range: 0.3-3.0cm). During a mean follow-up of 52.2 months (range: 16-118 months), all patients with benign tumor survived without recurrence. Among the 28 HB cases, 3 experienced recurrence, with 3- EFS and OS rates of 89.01% and 91.92%, respectively.

Conclusion: Laparoscopic major hepatectomy was safe and feasible for selected pediatric patients. Mid-term outcomes for HB laparoscopic major hepatectomy were satisfactory.

Abstract 252

Six-drug combination chemotherapy for parameningeal rhabdomyosarcoma with meningeal invasion: a single center prospective study

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Purpose: This study aimed to report the efficacy and safety of a six-drug combination chemotherapy regimen consisting of doxorubicin, carboplatin, ifosfamide, dactinomycin, vincristine, and etoposide in children with newly diagnosed PM-RMS with meningeal invasion(MI).

Methods: Patients with PM-RMS presenting with any of the following high-risk factors-cranial base bone erosion, intracranial invasion, cranial nerve palsy, or positive cerebrospinal fluid cytology-received 16 cycles of six-drug combination chemotherapy. The primary endpoints were best overall response rate(ORR), progression-free survival (PFS), and overall survival (OS). The secondary endpoint was the incidence of unacceptable toxicity.

Results: Between October 2018 and December 2022, a total of 52 patients with RMS met the inclusion criteria. The overall response rate was 93.9%. At data cutoff, 12 patients had died, 38 were alive, and 20 had developed recurrence or progression. The 3-year OS and PFS rates for the entire cohort (n=50) were 80.0%

and 67.4%, respectively.In the IRS group III (n=40), the 3-year OS was 82.5% and PFS was 75.0%. In IRS group IV (n=10), the 3-year OS was 70.0% and PFS was 40.0%.Multivariate analysis demonstrated that lymph node metastasis (HR=0.157, 95%CI 0.046 to 0.540, P=0.003) and intracranial extension (ICE) (HR=0.221, 95%CI 0.061 to 0.798, P=0.021) were risk factors for OS, and lymph node metastasis was a risk factor for PFS (HR=0.255, 95%CI 0.102 to 0.635, P=0.003).Among patients with ICE (n=24), one-third eventually developed leptomeningeal dissemination and died. Toxicity monitoring showed a high incidence of grade 3-4 myelotoxicity, while other grade 3-4 toxicities were rare.

Conclusion: Survival outcomes in this cohort were higher than those previously reported, suggesting that patients of PM-RMS with MI may benefit from intensive six-drug combination chemotherapy. Leptomeningeal dissemination is the major cause of mortality. It is necessary to establish a grading system for the severity of MI and feasible strategies for CNS prophylaxis.

Abstract 253

The Clinical Spectrum and Divergent Outcomes of Pediatric Extracranial Malignant Rhabdoid Tumor

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Purpose: Extracranial Malignant rhabdoid tumors (eMRTs) are aggressive pediatric cancers characterized by poor prognosis and the absence of standardized treatment protocols. This multicenter retrospective study aimed to analyze the clinical features, treatment patterns, and outcomes of pediatric eMRTs in China.

Methods: A retrospective analysis was conducted on 68 pediatric patients diagnosed with eMRT between 2017 and 2023 across nine tertiary medical centers in China. Patients were categorized into two subgroups: malignant rhabdoid tumor of the kidney (MRTK), and extracranial extrarenal rhabdoid tumor (EERT). Survival analysis was performed using Kaplan-Meier methods and log-rank tests.

Results: The median age at diagnosis was 19 months (range: 0-173 months). The cohort included 35 MRTK and 33 EERT cases. INI-1 loss was observed in 97% of cases, while two EERT cases demonstrated BRG1 deficiency. The 2-year overall survival (OS) for the entire cohort was 50.8%. Notable differences in outcomes were observed by tumor site, with EERT demonstrating a 2-year OS of 63.7% compared to 38.3% for MRTK. Advanced disease stage predicted worse outcomes in EERT patients but did not demonstrate prognostic significance in MRTK. Considerable heterogeneity was observed in treatment approaches, particularly regarding chemotherapy regimens.

Conclusion: Tumor site emerges as an important prognostic factor in pediatric eMRTs, with EERT showing superior outcomes compared to MRTK in this cohort. The lack of prognostic value for disease stage in MRTK may reflect the inherently aggressive biology of renal rhabdoid tumors in young children. The substantial treatment heterogeneity across centers underscores the urgent need for standardized, risk-adapted therapeutic protocols to improve outcomes for this devastating disease.

Abstract 254

Clinical Characteristics and Management of Postoperative Intestinal Obstruction in Neuroblastoma Patients: A Single-Center Retrospective Study

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Purpose: Gross total resection is often preferred for neuroblastoma despite the frequent involvement of abdominal organs and vessels. Consequently, postoperative intestinal obstruction (POIO) is a significant potential complication. This study retrospectively reviews our institutional experience regarding the clinical characteristics and management of POIO in neuroblastoma patients.

Methods: We retrospectively reviewed neuroblastoma patients admitted to our center between 2012 and 2021. Inclusion criteria included a confirmed neuroblastoma diagnosis and followed surgical tumor resection. POIO was defined as intestinal obstruction directly related to the surgical procedure. Exclusion criteria included incomplete medical records, obstruction unconfirmed by both symptoms and radiological findings, or vomiting/constipation attributed to chemotherapy or other non-surgical factors, such as physiological postoperative ileus.

Results: Of 719 patients diagnosed with neuroblastoma (542 INRGSS stage L2 or M), 509 underwent surgical resection. Fifteen patients (2.9%) developed POIO. Among these, 13 were INRGSS stage M, one was stage L2, and one was stage L1. The mean preoperative Image-Defined Risk Factors (IDRFs) score was 3.0 ± 1.3, and mean surgery duration was 5.5 ± 2.4 hours. POIO onset occurred at a median of 7.5 days (range: 3–45) post-surgery. Ten patients were managed conservatively, while five required surgical intervention. One patient died due to extensive small intestinal necrosis. Surgical interventions included temporary ostomy (n=1) and adhesiolysis (n=3). Notably, another one case of chronic abdominal distension persisting for 7 years after tumor resection was ultimately diagnosed as Hirschsprung's disease.

Conclusion: Postoperative intestinal obstruction is a rare but serious complication in neuroblastoma surgery, occurring predominantly in high-stage disease. While most cases resolve with conservative management, surgical intervention may be required. Clinicians should remain vigilant for underlying conditions, such as Hirschsprung's disease, in cases of chronic postoperative symptoms.

Abstract 255

Sequential intravenous and intracerebroventricular GD2-NKG2D multi-targeting CAR-T Therapy for CNS-relapsed/high-risk MYCN-amplified Neuroblastoma

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Purpose: Central nervous system (CNS) recurrence predicts dismal prognosis in high-risk MYCN-amplified neuroblastoma with limited treatments. We conducted a prospective trial (NCT06836505) at Sun Yat-sen University Cancer Center, evaluating sequential intravenous (IV) and intracerebroventricular (ICV) delivery of GD2-NKG2D multi-targeting CAR-T (a novel, third-generation, multi-targeting CAR-T product, DeepTag-GD2-NKG2D Bicephali, directed against both GD2 and NKG2D ligands) to prevent CNS recurrence in relapsed/high-risk patients.

Methods: Eligible patients received lymphodepletion followed by IV CAR-T (1×10^6 cells/kg). After IV adverse event (AE) resolution, ICV CAR-T was given every 4 weeks (dose-escalation: 1×10^7, 3×10^7, 5×10^7 cells, no lymphodepletion). Primary endpoints were safety and efficacy of the dual-administration strategy.

Results: Seven patients (median age 67.8 months, 5M/2F) were enrolled (Jan 2025–Feb 2026): 6 CNS-relapsed, 1 high-risk with MYCN-amplified. They received 8 IV and 29 ICV infusions. IV AEs were mainly grade 3–4 hematologic toxicities (100% neutropenia) and grade 1–2 cytokine release syndrome (CRS, 84.6%). ICV AEs were all grade 1 (CRS 72.4%, ICANS 44.8%). CSF CAR-T expansion peaked at 1.37% (median 29-day persistence). At median 6.0-month follow-up, no CNS recurrence occurred; only 1 patient had extracranial recurrence.

Conclusion: IV combined ICV GD2-NKG2D multi-targeting CAR-T therapy shows favorable safety and remarkable CNS disease control in relapsed/high-risk neuroblastoma. This promising strategy merits further clinical validation for this ultra-high-risk population.

Abstract 256

C18 GD2 LIQUID BIOPSY: A NEW PROGNOSTIC BIOMARKER IN LOW-RISK NEUROBLASTOMA PATIENTS?

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Purpose: Disialoganglioside GD2 is expressed on the plasma membrane of neuroblastoma (NB) cells and is shed into the bloodstream. The aim of this study was to evaluate circulating C18 GD2 as a prognostic biomarker in patients with low-risk NB.

Methods: A total of 101 patients with histologically confirmed low-risk NB according to GPOH NB2004 criteria were prospectively recruited. Concentrations of circulating GD2 (C18 lipoform) were measured in peripheral blood using high

performance liquid chromatography–tandem mass spectrometry (HPLC MS/MS) before and after therapy (surgery, chemotherapy).

Results: In localized NB patients (INSS stages 1–3, n=43), median preoperative GD2 level was 19.0nM(IQR 15.0–43.0) and decreased to 15.0nM(IQR 0.0–17.0) after surgery ($p < 0.001$). In stage 4S patients (n=7), median GD2 levels changed from 77.0(IQR 35.0–88.0) to 59.0(IQR 15.0–66.0)nM postoperatively ($p = 0.063$). Among localized stages, median GD2 decreased from 19.0(15.0–40.0) to 15.0(0.0–15.0)nM after macroscopically radical resection (n=27, $p<0.001$) and from 15.5(11.2–73.5) to 7.5(0.0–15.0) nM with residual macroscopic disease (n=10, p=0.02), biopsy (n=6) didn't significantly affect GD2 levels (38.0 [11.2–160.5] to 53.5 [15.0–81.5] nM; p=0.89). A cutoff of 28.3 nM, derived from ROC analysis, was used to define high (≥28.3 nM) and low (<28.3 nM) GD2 subgroups. Baseline GD2 concentrations before specific therapy didn't influence 3-year EFS (86.7%±6.3% vs 83.6%±6.8%; p=0.9). Elevated GD2 at completion of therapy was associated with inferior 3-year EFS in patients treated with surgery alone (89.5%±5.0 vs 47.0%±15.0; p=0.009), in those receiving combined modality treatment (88.9%±10.5% vs 50.0%±20.4%; p=0.15), and in the overall cohort (89.5%±4.5 vs 48.0%±12.5%; p=0.002). In univariable Cox regression, circulating GD2 level at the end of therapy was significantly associated with EFS (p=0.012; HR 1.010; 95% CI 1.002–1.018).

Conclusion: Circulating C18-GD2 may be used as a prognostic marker for low-risk NB patients, as a liquid biopsy method. Studies on a larger cohort of patients are needed to validate the current results.

Abstract 257

Apatinib Combined with Irinotecan and Temozolomide for Relapsed or Refractory Neuroblastoma (AIT-NB-01): A Multicenter, Single-Arm, Phase I/II Clinical Study

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Purpose: Relapsed or refractory neuroblastoma (r/r NB) is associated with a dismal prognosis and limited treatment options. We conducted a multicenter phase I/II study to evaluate the efficacy and safety of apatinib combined with irinotecan and temozolomide (AIT) in patients with r/r NB.

Methods: Patients with r/r NB and measurable diseasewere eligible. Patients received apatinib combined with 5-day courses of irinotecan (50 mg/m²/day) and temozolomide (150 mg/m²/day). Phase I employed a 3+3 dose-escalation (apatinib: 150, 300, 450 mg/m²) to determine RP2D. In phase II, patients received up to eight cycles of AIT followed by apatinib maintenance until progression or unacceptable toxicity. Primary endpoint was objective response rate (ORR). Secondary endpoints included safety, progression-free survival (PFS), and overall survival (OS).

Results: Between September 2021 and September 2024, 23 and 88 patients were enrolled in phase I and phase II, respectively. The median age at enrollment was 6.6 years (range, 1.3-52.4). Dose-limiting toxicity observed at 450 mg/m² established the RP2D at 300 mg/m². Among 96 patients treated at the RP2D, the ORR was 54.2% (18 CR, 34 PR). Relapsed/progressive patients showed a higher ORR than refractory patients (67.2% versus 24.1%, P=0.00013). At a median follow-up of 26.5 months, median PFS was 11.7 months; median OS was not reached; 1-year PFS and OS rates were 47.4% (95% CI: 38.3-58.6) and 91.3% (95% CI: 85.7-97.3), respectively. Thirty-five patients entered the apatinib monotherapy maintenance phase, with a median maintenance duration of 7.7 months (range, 0.4-32.6). Apatinib maintenance therapy significantly prolonged PFS (median: 16.6 versus 10.6 months, P=0.047). Apatinib-related adverse events were manageable, including hypertension (2.9%) and hand-foot syndrome (0.67%).

Conclusion: The AIT regimen demonstrates promising efficacy with a manageable safety profile in patients with r/r NB, with an RP2D of apatinib at 300 mg/m². Apatinib maintenance therapy confers a significant survival benefit.

Abstract 258

Neoadjuvant therapy of juvenile nasopharyngeal angiofibroma

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Purpose: Juvenile angiofibroma of the skull base and nasopharynx (juvenile nasopharyngeal angiofibroma, JNA) is a benign neoplasm. It accounts for 0.5% of all tumors of the head and neck and occurs in 1 in 150 thousand people. The main direction in surgical managment of JNA is searching a method to reduce the risk of massive bleeding during operations and to reduce the risk of recurrence or progression of JNA in the postoperative period.

Methods: The prospective open non–randomized interventional study with a historical control group in accordance with the international standard of ethical standards and quality of scientific research - GCP (good clinical practice) was conducted at NMRC of PHOI named by Dmitry Rogachev since 30 may 2022.

Results: 231 patients have received surgical removal of JNA at NMRC of PHOI named by Dmitry Rogachev. Surgical treatment of patients with advanced stages of JNA, like IIIa or higher according to the Fisch-Andrews classification, is associated with high risks of surgical treatment due to the development of massive intraoperative bleeding despite the preoperative selective embolization of the supplying arteries. Currently the pilot phase of the study has been completed with the achievement of control points. The main phase of the study has begun in 15 patients with primary JNA. The recruitment of patients with recurrent angiofibroma for the pilot phase of the study is still ongoing. At the moment, 14 patients out of 15 patients with primary JNA in the pilot phase of the study have managed to achieve a reduction of tumor volume of more than 20%. In a number of several patients with recurrent JNA, surgical treatment was avoided in due to the absence of complaints of impaired nasal breathing and nosebleeds.

Conclusion: Surgical treatment of juvenile angiofibroma of the skull base is currently the method of choice, especially for patients with primary angiofibroma with a small tumor volume and with the stage I-II of JNA according to the Fisch-Andrews classification. By now, the study of interest is the effectiveness of targeted therapy with mTOR inhibitors in the management of JNA , however, the efficacy and safety of Sirolimus requires further study.

Abstract 259

Clinical Significance of Bone Marrow Disseminated Tumor Cells in Pediatric Neuroblastoma: A Prospective Observational Study

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Purpose: Despite multimodal therapy, 5-year EFS for high-risk neuroblastoma (NB) remains <60%, largely due to minimal residual disease (MRD). Here we used DLD microfluidic chip to isolate bone marrow disseminated tumor cells (DTCs) and clusters, assessing their clinical and prognostic relevance.

Methods: We enrolled 80 newly diagnosed NB patients from Jan 2020–Dec 2024. Bone marrow (2 mL) was collected at diagnosis and during treatment. DTCs/clusters were enriched via DLD chip and identified by GD2/PHOX2B immunofluorescence. Parallel flow cytometry-based MRD (FCM-MRD) was performed.

Results: DTC/DTC cluster presence strongly correlated with metastasis, advanced INSS/INRG stage, and high risk. DTC levels declined with therapy response. Cox analysis confirmed DTC (HR=2.41) and cluster (HR=2.78) positivity as independent 3-year EFS risk factors.

Conclusion: Bone marrow DTCs and clusters offer superior sensitivity over conventional MRD or biopsy for minimal disease detection. They enable early high-risk identification and predict relapse 4–8 weeks before MRD positivity. Combined with imaging and FCM-MRD, dynamic DTC monitoring provides a refined framework for personalized neuroblastoma management.

Abstract 260

TPOG NB 2020 Protocol: Results of High-Risk Group Conventional Chemotherapy Arm and the Impact of Immunotherapy as Maintenance Treatment

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Purpose: To evaluate results of high risk group conventional chemotherapy arm of national protocol

Methods: Clinical characteristics, treatment outcomes and survival rates of 300 patients were analysed.

Results: Median age at diagnosis was 29 months (0-17 years). Primary tumor locations were;surrenal (59%), paravertebral (26%), midline (13%), multifocal (2%). Metastatic disease was seen in 72% of the patients involving bone (54%), bone marrow (53%), distant lymph node (22%) liver (13%), lung (8%) and CNS (6%). MYCN amplification was detected in (64%),11q deletion in (40%). Stage distribution was L1 (10%), L2 (18%), M (69%), MS (3%). Primary surgery performed on 24% of the patients. Following 3 courses of chemotherapy, complete/partial response achieved in 79 % (n:258). After 6 courses overall response rate was 86%. Delayed surgery was performed in 50% of patients. Radiotherapy administered on 148 patients. Maintenance therapy applied to 163 patients. Immunotherapy was given to 39% of patients, 61% of them were on chemotherapy maintenance arm. For all patients EFS rates were 59%, 40% and 37% in 1,3 and 5 years; and OS rates were 90%, 71% and 66% at 1,3 and 5 years respectively. In the immunotherapy maintenance arm EFS rates were 79% for 2 years and 71% for 3 years. In the chemotherapy maintenance arm EFS rates were 62% for 2 years and 54% for 3 years. Difference in two arms was significant (p=0.023). In the immunotherapy arm OS rates were 96% for 2 years and 89% for 3 years. On chemotherapy arm OS rates were 84% for 2 years and 76% for 3 years. Difference in two arms was significant (p=0.029).

Conclusion: Usage of immunotherapy as the maintenance treatment in the conventional chemotherapy arm of high risk patients caused statistically significant increase in EFS and OS rates in comparison with previous national protocol.

Abstract 261

Molecular genetic landscape in pediatric patients with pheochromocytoma/paraganglioma syndrome: a single-center experience

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Purpose: Pheochromocytoma/paraganglioma syndrome (PPGLS) is a heterogeneous group of genetic conditions characterized by predisposition to various tumors, most frequently to neuroendocrine neoplasms during the lifetime. This study presents the results of molecular genetic testing and clinical presentation in a cohort of pediatric patients with PPGLS in our Center.

Methods: A cohort of 46 pediatric patients (aged 10 months–18 years) with genetically confirmed PPGLS was retrospectively analyzed. Germline only or combined somatic and germline testing were performed using NGS, MLPA analysis and Sanger sequencing, with the optimal approach selected based on the specific clinical context.

Results: Among 46 patients with PPGLS, there were 43 probands and 3 asymptomatic siblings. We identified three groups. Group 1 (38 probands) had typical PPGLS tumors, the most common tumor was paraganglioma (20/43, 46.5%), followed by pheochromocytoma (7/43, 16%). Group 2 (2 probands) had an atypical tumor for PPGLS (neuroblastoma) in this group, tumor tissue was tested, and PPGLS was subsequently confirmed by blood DNA testing. Group 3 (3 probands) had various diseases (immunodeficiency, cherubism, and atypical teratoid/rhabdoid tumor of the CNS), with PPGLS identified incidentally. PVs were most frequently identified in the SDHB gene (24/43, 56%), followed by VHL gene (9/43, 21%), SDHD gene (5/43, 12%), SDHC gene (2/43, 5%), SDHA gene (1/43, 2%), and TMEM127 gene (2/43, 4%). In one unique case we detected a rare complex substitution at the donor splice site of SDHB (SDHB:c.[765+1G>T];[765+3A>T]).

Conclusion: PPGLS can be genetically confirmed not only in patients with paraganglioma and pheochromocytoma but also in cases with other tumors or as a secondary finding.

Abstract 262

Efficacy of reduced doses of melphalan in nephroblastoma: date from retrospective trial from two centers

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Purpose: High-risk Wilms tumor (WT) requires autologous hematopoietic stem cell transplantation (aHSCT) as a part of treatment plan. High-dose chemotherapy with melphalan is associated with high mortality and morbidity. We presenting retrospective analysis of children with WT received aHSCT in different dosing regimen of melphalan.

Methods: A retrospective study analyzed the results of treatment of 27 children with WT (M/F ratio 15/12) belonging to the group of extremely high risk (group D, n = 3, 11.1%), disease progression/recurrence (n=24, 88.9%). Nineteen patients (70.4%) received two lines of induction, and 5 (18.5%) three or more lines of therapy (SIOP RTSG 2016 protocol). Median of age was 6 years 5 months. Peripheral blood stem cell apheresis (PBSC) was performed in all patients: 24 (89%) patients required one apheresis, 3 (11%) two or more. Full dose of Melphalan (single dose of 200 mg/sq.m.) was used in 17 (63.0%) cases (group 1), and reduced dose (140 mg/sq.m.) – in 10 (37.0%) cases (group 2). The median of infused CD34+ cells was 4.9 × 10 6/kg.

Results: All patients engrafted. Median of neutrophils recovery were faster in group 2 (7 days) in the contrast of group 2 (17 days). Toxicity rates were same in both groups, but no mucositis of gr. 3-4 was found in group 2. With a median follow-up of 1.5 years, 19/27 patients are alive in both groups. Overall survival: group 1 – 43.0%, group 2 – 85.7%. Disease-free survival: group 1 – 36%, group 2 – 78%. Causes of mortality in group 1: toxicity – 4, progression – 2; in group 2: toxicity – 1, progression – 1.

Conclusion: Autologous HSCT is an effective method of consolidation in case of WT of unfavorable prognosis in children. Our study showed that reduction of doses of Melphalan improves both overall and disease-free survival. Future large multicenter trials required.

Abstract 263

Toxicity of treosulfan and busulfan in autologous peripheral stem cell transplantation in neuroblastoma and Ewing sarcoma.

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Purpose: Autologous hematopoietic stem cell transplantation (aHSCT) is effective way of consolidation therapy in neuroblastoma (NB) and Ewing sarcoma (ES). Balance between toxicity and efficacy is important. We aimed to present the data of retrospective analysis from 2 centers describing toxicity and efficacy of aHSCT for the patients with NB and ES.

Methods: Our retrospective study includes 148 patients with NB (n=116, 78%) and ES (n=32, 22%). M:F=62(46%):86(54%). All patients received aHSCT with treosulfan/melphalan or busulfan/melphalan conditioning in 2021-2023. Age median was 5.1 y.o. [8 months – 17 y.o.]. The majority of patients (73.6%) received aHSCT as a part of first-line therapy with complete response in 72.9% cases. HSCT with Treosulfan-based conditioning regimen received 89 patients (60.1%) and Busulfan-based – 59 (39.9%). Median infused dose of CD34+ cells was 7.110 6 /kg. Toxicity as well as overall survival and relapse-free survival estimated.

Results: All patients engrafted. Median day of engraftment was better in treosulfan group (11 vs 15). Toxicity rates were better in treosulfan group both mucositis and neutropenia: grade 3-4 – 12% in treosulfan group and 25% in busulfan group (p<0.005). Toxic deaths: treosulfan: n=2 (2.24%) and busulfan n=2 (3.4%). No differences were found in nutrition insufficiency, viral reactivations and hemorrhagic complications. At the median of FU of 2 years OS was higher in treosulfan group (78.1% vs 73.1%) as well as RFS (65.3% vs 59.8%), but no statistical difference found.

Conclusion: Treosulfan-based conditioning regimen is safe and effective for NB and ES. Less organ toxicity found in patients with treosulfan-based conditioning regimen in comparison with busulfan-based conditioning. Large multicenter studies required.

07. SOLID TUMORS — POSTER

Abstract 264

BEYOND PRETEXT: EXPLORING BIOLOGIC AND DYNAMIC DETERMINANTS TO REFINE RISK IN CHILDREN WITH HEPATOBLASTOMA- A SINGLE CENTER EXPERIENCE FROM INDIA

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Purpose: PRETEXT staging was designed for surgical planning and risk stratification in pediatric hepatoblastoma. However, survival outcomes often vary within similar anatomical stages suggests that additional determinants may influence outcomes. Present study aimed to evaluate whether biologic risk features and early dynamic treatment response provide additional prognostic insight beyond baseline PRETEXT staging in a real-world.

Methods: A retrospective observational study was conducted in 37 children diagnosed with hepatoblastoma between 2021 and 2025 at tertiary cancer center in western India. Baseline demographic details, clinical presentation, PRETEXT stage, annotations, alpha-fetoprotein (AFP) details, radiologic findings, chemotherapy details, vascular involvement and metastasis status were recorded. Disease-free survival (DFS) and overall survival (OS) were analyzed using univariable cox regression and Kaplan–Meier method. Dynamic response was evaluated using post-neoadjuvant chemotherapy (NACT) AFP decline in conjunction with post NACT-CT scan response compare to baseline.

Results: OS was observed in 18 (49%) patients. PRETEXT stage significantly impact surgical feasibility (p=0.03) but did not independently distinguish outcome. Higher annotation burden predicted inoperability (p=0.04) with inferior outcomes was observed in portal venous involvement (HR 2.76 for DFS, p=0.032) and tumor rupture (HR 3.90, p=0.046). Metastasis was strongly associated with mortality (91% vs 35%, p=0.01). Surgical intervention was associated with improved DFS (HR 0.17, p=0.004) and OS (HR 0.28, p=0.027). Dynamic response outperformed baseline AFP (AUC 0.673 vs 0.491) and showed favourable associations with surgery and outcome.

Conclusion: PRETEXT staging guided operability but did not fully discriminate survival, highlighting the importance of incorporating adverse biologic features of tumor and dynamic treatment response when interpreting risk in children with hepatoblastoma. These observation findings should be further explored in larger, multicentric trails.

Abstract 265

Clinical Outcomes of Pediatric Rhabdomyosarcoma According to the 2022 EpSSG Risk Stratification: A Single-Center Study from K Hospital, Vietnam.

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Purpose: Rhabdomyosarcoma is a common pediatric malignancy at K Hospital, accounting for 24 of 428 cancer cases (5.6%) in patients aged 0–19 years during 2023–2024. Most patients present with high- or very high-risk disease. Aim: To evaluate survival outcomes of pediatric rhabdomyosarcoma treated at K Hospital.

Methods: We conducted a cross-sectional, retrospective study with totally 24 newly-diagnosed children in the year of 2023 and 2024. The data cut-off date for analysis was March 15th 2026. Patient characteristics and survival outcomes were retrospectively analyzed.

Results: A total of 24 patients with rhabdomyosarcoma (RMS) were diagnosed and treated. The mean age at diagnosis was 9.92 years (range 1–18 years) and most patients were male (n=19, 79.2%). The median follow-up was 30.9 months. According to the European Paediatric Soft Tissue Sarcoma Study Group (EpSSG) risk classification, there were n=1 with standard group, n=15 with high risk group and n=8 (33.3%) with very high-risk group. FOXO1 amplification was absent in 15/24 patients (62.5%). Histologically, 13 patients were embryonal RMS, 9 were alveolar RMS, and 2 had spindle cell/sclerosing RMS. The primary sites of tumour, there were: n=11 of the head and neck (including 7 parameningeal), n= 9 of the abdomen (including 2 genitourinary sites), and n=4 of the extremities. At the time of analysis (15 March 2026), 14 patients (58.3%) were alive. Median progression-free survival (PFS) is 20.4 months and the overall survival (OS) is 21.1 months. Objective Response Rate (ORR) reached at 20 patients equivalent to 80%. Comparison of PFS and OS between metastatic disease and node-positive disease with detected FOXO fusion showed no significant difference.

Conclusion: RMS still represents a persistent challenge in both diagnostic precision and therapeutic management. However, advances in modern treatment strategies have substantially improved survival outcomes.

Abstract 266

Changing Patterns of Wilms Tumor Presentation and Surgical Approach Over 25 Years: Insights from a National Pediatric Oncology Center Toward Childhood Cancer Equity

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Purpose: Wilms tumor is the most common renal malignancy in childhood, and survival outcomes are strongly influenced by stage at diagnosis. In many health systems, delays in recognition and referral contribute to advanced-stage presentation, reflecting broader disparities in childhood cancer care. Long-term institutional data from national referral centers can provide important insights into diagnostic pathways and evolving treatment strategies.

Methods: We conducted a retrospective cohort study of children diagnosed with Wilms tumor at the national pediatric oncology center “Mother Theresa University Hospital Center”, Tirana, over a 25-year period. Demographic and clinical data were reviewed, including age and stage at diagnosis, interval from symptom onset to diagnosis and surgical approach. Trends in stage distribution and surgical management were analyzed over time.

Results: A total of 98 patients were included. The estimated national incidence was 8.34 cases per million children per year. The median age at diagnosis was 3 years. Stage III disease represented the most frequent stage at presentation, followed by Stage II. Early-stage disease accounted for a smaller proportion of cases. Decade-based analysis points a gradual shift toward earlier-stage presentation in recent years, reflecting improvements in referral pathways and diagnostic. The reported median interval from symptom onset to diagnosis was approximately one week, although many children had very large abdominal masses, suggesting longer diagnostic interval with advanced-stage tumors having longer delays. Front-line surgery remained the preferred treatment strategy in our cohort in 64% of patients, while neoadjuvant chemotherapy was reserved for cases where surgery was not feasible due to advanced stage.

Conclusion: This 25-year experience highlights both the persistence of advanced-stage presentation and encouraging signals of progress toward earlier diagnosis. Strengthening early recognition, referral pathways, and pediatric oncology networks remain essential in reducing diagnostic delays and advancing equity outcomes.

Abstract 267

First experience with dinutuximab-beta in children with high-risk neuroblastoma: treatment results from a single center in Kazakhstan

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Purpose: Neuroblastoma (NB) is the most common malignant extracranial solid tumor in childhood, with at least 60% of cases falling into the high-risk group. Among malignant neoplasms in the Republic of Kazakhstan, NB ranks third, accounting for 6.4%. Up to 25 primary patients are hospitalized annually at the NCPDS, of which 43% are in stages I-II and 57% in stages III-IV. The 5-year survival rate is 54%.

Methods: Between 2022 and 2024, 11 patients were treated with dinutuximab-beta. Eight (73%) of these patients received the full protocol course of therapy—five cycles of dinutuximab-beta. Two (18%) children were discontinued during the immunotherapy stages due to disease progression. The parents of one (9%) child declined further treatment.

Results: Of the eight patients who received the full course of immunotherapy, five (62.5%) responded. All five children are currently under dynamic observation. According to the prescribed examination schedule (contrast-enhanced MRI, PET/CT), there has been no progression. The observation period is 32-18 months (two children have been observed for 32-30 months, and three children have been observed for 22-18 months). Of the 11 children, 5 (45.5%) died due to disease progression after several courses of alternative therapy and due to the inability to resume immunotherapy (due to drug unavailability). During therapy, no serious adverse events or deaths related to dinutuximab-beta were observed. Expected adverse events included moderate hematological toxicity, which in some cases led to delayed repeat drug administrations.

Conclusion: It is important to note that for patients with high-risk stage IV neuroblastoma, dinutuximab-beta offers a chance for survival and improved quality of life, as survival at this stage was previously limited to 6 months.

Abstract 268

Improved B cell function is Associated With Better Disease Control in High-Risk Neuroblastoma Patients Receiving Allogeneic Hematopoietic Stem Cell Transplantation

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Purpose: High-risk neuroblastoma (HR-NB) is a highly intractable disease requiring aggressive therapy. In recent years, allogeneic hematopoietic stem cell transplantation (allo-HSCT) has been tried in the treatment of HR-NB. However, there is limited real-world data for immune reconstitution and its impact on disease control in HR-NB patients after allo-HSCT.

Methods: We conduct a retrospective cohort study evaluating quantitative hematologic and immune subsets in 14 patients with HR-NB underwent allo-HSCT. The immune status in different periods after allo-HSCT were compared and differences of immune status in patients with or without recurrence after allo-HSCT were analyzed.

Results: Compared to those without disease recurrence, patients with disease recurrence had significantly lower absolute B cell counts at 3 months 91.71 vs 444.86 cells/ul, P=0.036), lower Treg cell counts at 3 months (15.61 vs 30.03 cells/ul, P=0.008) , lower serum levels of C3 at 6 months (951.17 vs 1107.5 cells/ul, P=0.043) and high serum IgA level at 12 months after allo-HSCT (0.68 vs 0.37 cells/ul, P=0.022) .

Conclusion: Improved B cell function is associated with better disease control in HR-NB patients receiving allo-HSCT.

Abstract 269

The Utility of D-Dimer and Fibrinogen Levels as Markers for Diagnosis, Treatment Response, and Prognosis in Pediatric Solid Tumors

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Purpose: Systematic activation of hemostasis, independent of thrombosis, can be observed in childhood solid tumors. This study investigated the utility of D-dimer and fibrinogen parameters at initial diagnosis, for evaluating prognosis, and in relation to metastasis in pediatric patients with solid tumors.

Methods: A retrospective study was conducted at the Ankara City Hospital Pediatric Oncology Clinic, initially screening 185 patients aged 0-18 years with solid tumors. Patients evaluated for fibrinogen and D-dimer levels at diagnosis and subsequent follow-ups were included. Patients presenting with thrombosis or secondary conditions (e.g., infections) that could affect the parameters were excluded. Ultimately, the clinical data of 75 patients were analyzed.

Results: The primary malignancies were bone tumors (n=32), neuroblastoma (n=16), and rhabdomyosarcoma (n=13). At the time of diagnosis, 36 patients had metastases. The median age was 7 years (range: 0-17). Nine patients died during the follow-up period, and the mean overall survival was 43.0±2.3 months. ROC curve analysis demonstrated that a diagnostic D-dimer value >1.31 predicted mortality with 100.0% sensitivity and 56.9% specificity (AUC: 0.727). Diagnostic D-dimer showed a moderate positive correlation with diagnostic LDH (r=0.558), CRP (r=0.358), ESR (r=0.428), and ferritin (r=0.534). Diagnostic fibrinogen positively correlated with diagnostic CRP (r=0.500) and ESR (r=0.374). At the end of treatment, D-dimer moderately correlated with final LDH and fibrinogen , while final fibrinogen correlated with final CRP, ESR, and D-dimer. Although LDH levels significantly decreased across treatment phases (p=0.011) , D-dimer and fibrinogen levels did not show statistically significant variation over time. Additionally, D-dimer and fibrinogen levels showed no significant differences between patient groups with and without metastasis (p>0.05).

Conclusion: These findings suggest that D-dimer and fibrinogen levels have significant potential to be utilized as clinical markers in childhood solid tumors. Furthermore, the D-dimer level at the time of initial diagnosis is strongly associated with the predictability of mortality, offering a valuable tool for early risk stratification.

Abstract 270

Bone Marrow Immune Microenvironment in High-Risk Neuroblastoma During Anti-GD2 Immunotherapy

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Purpose: Neuroblastoma (NB) is the most common extracranial solid tumor in children and frequently presents with bone marrow (BM) metastases. Despite intensive multimodal treatment, outcomes for high-risk NB patients remain poor. The immune microenvironment of metastatic BM may influence the efficacy of anti-GD2 immunotherapy (IT). This study aimed to characterize BM lymphocyte subsets in NB patients and assess their dynamics during IT.

Methods: Twenty-one NB patients receiving dinutuximab beta IT were prospectively enrolled. BM samples were analyzed using multicolor flow cytometry (FACSCanto II, BD Biosciences, USA) at three time points: before IT, during treatment, and after therapy completion. Patients were stratified according to the presence or absence of BM involvement. Intergroup comparisons were performed using the Mann–Whitney test, while longitudinal changes were assessed using linear mixed-effects models. NK-cell functional status was evaluated using activation (CD57, CD160, CD314) and exhaustion (Tim-3, CD159a) scores. The NK/Treg ratio was calculated as a measure of the balance between NK-mediated cytotoxicity and regulatory T-cell-mediated immunosuppression.

Results: At baseline, patients with BM involvement demonstrated significantly higher levels of Tim-3-expressing NK cells (p=0.0115). Significant differences were also observed in NK activation (p=0.049) and exhaustion (p=0.008) scores.

In contrast, the NK/Treg ratio did not differ between groups (p=0.981). Longitudinal analysis revealed significant differences in the dynamics of Treg subsets CD294+CD45RA- and CD294-CD45RA+ during therapy. No significant differences were observed in NK-cell subset dynamics between the groups.

Conclusion: These findings suggest that BM metastatic involvement in neuroblastoma is associated with alterations in NK-cell functional status, particularly in markers related to immune activation and exhaustion. Further investigation in larger cohorts may help clarify the role of BM immune microenvironment in the response to anti-GD2 IT.

Abstract 271

Difluoromethylornithine (DFMO) maintenance in patients with very high-risk neuroblastoma

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Purpose: Neuroblastoma (NB) ist the most common extracranial solid tumor in infants characterized by extreme biological heterogeneity. In spite of the use of intensive complex treatment regimens the long-term survival in patients with most adverse prognosis hardly exceeds 50%. There is also no uniformly accepted standard for relapsed and refractory disease and even reinduction therapy responders tend to develop a further relapse. The long-term maintenance therapy may be a feasible option and polyamine synthesis pathway inhibitors Difluoromethylornithine (DFMO) have favorable toxicity profile and were shown to be effective in several clinical trials. We aim to describe the first clinical experience of DFMO maintenance therapy in Russian Federation.

Methods: A total of five patients with very high-risk NB received DFMO maintenance. The median age at therapy initiation was 6 (range 5-10) years. One patient was scheduled for DFMO therapy in spite of complete response to initial therapy due to multiple risk factors (initial age and metastatic burden, multiple biological risk factors). Two patients had multiple residual MIBG-avid lesions after several lines of therapy. One patient (initially high-risk) previously developed a 1st late systemic relapse achieving a partial response after several therapy lines including allogeneic hemopoietic stem cell transplantation from haploidentical donot (haplo-HSCT). Another patient, initially from observation group, developed a second systemic relapse resonding well to complex therapy (including haplo-HSCT as consolidation). Oral DFMO was given in standard dose regimen (2 to 3 192 mg tablets depending on body surface area).

Results: With a median follow-up of 6 (4-18) months none of the patients experienced significant treatment toxicity. None of the patients has currently signs of disease progression. In one case the positive dynamics (fewer MIBG-avid lesions) was registered.

Conclusion: DFMO has a favorable toxicity profile making it a suitable maintenance option even in severely pre-treated patients (e.g. haplo-HSCT recipients). None of the small cohort described has yet shown signs of disease progression in spite of multiple risk factors involved. However, there is still a need for further research, preferably as part of a multicenter study.

Abstract 272

Clinical Analysis of Neurofibromatosis Type I Combined with Malignant Tumors in Children

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Purpose: To summarize the clinical features, genetic characteristics and prognosis of children with neurofibromatosis type I (NF1) combined with malignant tumors.

Methods: The clinical data, genetic characteristics and prognosis of children diagnosed and treated with follow-up in our hospital with NF1 combined with malignant tumors were retrospectively analyzed.

Results: A total of 16 children were included in the analysis. There were 9 males and 7 females, with a median age of 3.9 years. The tumors included 5 cases of neuroblastoma, 7 cases of rhabdomyosarcoma, 1 case of hepatoblastoma, 1 case of PNET, 1 case of rhabdoid tumor, and 1 case of leukemia. Some of the children had tumor-related mutations other than NF1, such as TP53 mutations. All 16 children received chemotherapy. Except for leukemia, the remaining 15 children underwent surgical treatment, 8 received radiotherapy, 4 received immunotherapy, and 4 received targeted therapy. The median follow-up time was 21.5 months. 11 children survived, and 5 cases relapsed.

Conclusion: NF1 can be combined with multiple malignant tumors. Early identification of NF1 combined with malignant tumors and standardized treatment are very important.

Abstract 273

Ferric citrate and Arsenic Trioxide: A Potent Duo Against Neuroblastoma Through Ferroptosis Activation

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Purpose: Iron exhibits a close association with neuroblastoma (NB), as elevated serum ferritin is frequently observed in high-risk NB and correlates with an unfavorable prognosis. Ferroptosis, a recently identified cell death modality characterized by iron-dependent lipid peroxidation, involves the crucial triggering role of intracellular free iron overload.

Methods: In this study, we assessed cell viability, cellular morphology, and reactive oxygen species (ROS) levels in response to ferric citrate (FAC), arsenic trioxide (ATO), and their combination in NB cell lines SK-N-AS and SH-SY5Y. Subsequently, a rescue assay was employed to confirm that the primary mode of NB cell death induced by ATO in conjunction with FAC was ferroptosis.

Results: FAC augmented the inhibitory effects of ATO on NB cells. Increasing FAC concentrations did not significantly promote the proliferation of SK-N-AS and SH-SY5Y NB cell lines. The inhibitory impact of ATO on the proliferation of these NB cell lines was more pronounced with elevated ferroptosis indicators, specifically ROS, in combination with FAC. Moreover, treatment with the ferroptosis inhibitor Fer-1 alleviated the inhibitory effects of ATO combined with FAC on the two NB cell lines.

Conclusion: Our findings suggest that FAC does not notably contribute to tumor growth but enhances the inhibitory effects of ATO on NB cells. This indicates the potential of FAC combined with ATO as a promising treatment strategy for NB.

Abstract 274

Senescent Neuroblastoma Promotes M2 Macrophage Polarization and Tumor Progression through AA/GDF15 Pathway

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Purpose: Neuroblastoma (NB) is characterized by significant clinical heterogeneity, with outcomes ranging from spontaneous regression to aggressive recurrence. While chemotherapy induces direct cell death, it also triggers cellular senescence by damaging genomic DNA or inhibiting essential mitotic kinases required for cell division. Senescent NB cells undergo complex metabolic reprogramming and secrete various senescence-associated secretory phenotype (SASP) factors, which collectively remodel the tumor immune microenvironment to facilitate tumor progression. This study investigates the mechanisms by which senescent NB cells drive progression and identifies specific metabolic targets to enhance the efficacy of subsequent immunotherapy.

Methods: A senescent NB model was established using MLN8237 in vitro. Conditioned medium (CM) from these cells was co-cultured with THP-1-induced M0 macrophages to evaluate polarization. A xenograft model was established by co-injecting normal and senescent NB cell lines to assess tumor growth, while flow cytometry analyzed macrophage phenotypic changes within the immune microenvironment. Multi-omics sequencing was employed to identify altered metabolic pathways and related SASP factors in senescent NB cells.

Results: Flow cytometric analysis confirmed that senescent NB CM significantly upregulated the M2 macrophage marker CD206. In vivo experiments demonstrated that co-injecting senescent cells with normal cells accelerated NB progression. Multi-omics results identified the arachidonic acid (AA) metabolic pathway as a key driver. Correlation analysis confirmed that AA metabolites function through the GDF15 factor; specifically, AA metabolites promoted GDF15 secretion, and recombinant human GDF15 induced M2 polarization.

Combination therapy involving chemotherapy and an AA pathway inhibitor effectively suppressed NB progression and significantly ameliorated the immunosuppressive microenvironment.

Conclusion: Senescent neuroblastoma cell promotes M2 macrophage polarization and tumor progression via the AA/GDF15 signaling pathway. Combining chemotherapy with an arachidonic acid metabolic pathway inhibitor effectively inhibits neuroblastoma progression and offers a promising therapeutic strategy for enhancing the efficacy of clinical immunotherapy protocols.

Abstract 275

Clinical Characteristics and Prognosis of TP53-Mutated Pediatric Solid Tumors Based on Arsenic Trioxide Rescue Activity Stratification A 16-Case Analysis

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Purpose: TP53 mutations are associated with aggressive biology and poor outcomes in pediatric solid tumors. Recently, a stratification system based on Arsenic Trioxide (ATO) rescue activity (Type 1: Fully rescuable; Type 2: Partially rescuable; Type 3: Unrescuable) was proposed by Lu et al. (Sci Transl Med, 2023). This study aims to characterize the clinicopathological features of TP53-mutated pediatric solid tumors in our cohort and validate the prognostic value of this ATO-based stratification.

Methods: We prospectively enrolled 16 pediatric patients diagnosed with TP53-mutated solid tumors between 2018 and 2023 at Sun Yat-sen Memorial Hospital. Clinical data, genomic mutation profiles, and survival outcomes were collected. Patients were stratified according to the ATO rescue activity criteria. We analyzed the correlation between ATO stratification, specific mutation domains, co-mutations, and clinical outcomes.

Results: The median age of the 16 patients was 7.5 years. The tumor spectrum included sarcomas (5 cases), neuroblastoma (3 cases), and central nervous system tumors (3 cases). Half of the patients (8/16, 50%) presented with advanced-stage disease (Stage III/IV). With a median follow-up of 24 months, the progression/relapse rate was 50% (8/16), and the mortality rate was 31.3% (5/16). Hotspot mutations in the DNA-binding domain (e.g., R248, R273, R282) were identified in 31.3% (5/16) of patients and were associated with a 60% progression rate; however, this did not result in a statistically significant reduction in median Overall Survival (OS) (HR=2.8, p=0.15). Regarding ATO stratification, the Type 3 group (8 cases) showed a median OS of 22 months, compared to the Type 1-2 group (7 cases) where median OS was not reached. While the Type 3 group demonstrated a trend toward poorer survival (HR=2.1), the difference was not statistically significant in this cohort (p=0.29). Notably, co-mutation analysis revealed that activation of the PI3K pathway or MYCN amplification in Type 3 patients was strongly associated with adverse outcomes, with a median OS of only 18 months in deceased cases.

Conclusion: TP53-mutated pediatric solid tumors are characterized by early onset, advanced stage at presentation, and strong treatment resistance. Both DNA-binding domain hotspot mutations and ATO Type 3 stratification are associated with unfavorable prognosis. Although the ATO rescue activity stratification system did not reach statistical significance in this small cohort, it demonstrated a clear trend in risk stratification. These findings support the potential utility of this system in guiding future precision therapy strategies, particularly for identifying high-risk subgroups (e.g., Type 3 with PI3K/MYCN co-alterations) who may require intensified or novel therapeutic approaches.

Abstract 276

Congenital Bilateral Adrenal Neuroblastoma with Pepper Syndrome: An Autopsy Case Report

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Purpose: Neuroblastoma is an embryonal malignancy arising from neural crest–derived cells and represents one of the most common cancers in neonates and infants. Approximately 20% of cases are diagnosed prenatally or during the first months of life. A specific neonatal presentation, known as Pepper syndrome, is characterized by an adrenal primary tumor with extensive hepatic metastasis. Congenital neuroblastoma and bilateral adrenal involvement are uncommon.

Methods: Case presentation: We report a rare case of congenital neuroblastoma in a 1-month-12-day-old female infant. A large intra-abdominal mass was detected prenatally at 35 weeks of gestation. Postnatal computed tomography revealed marked hepatomegaly with multiple hepatic lesions and a mass involving the right adrenal gland. Chemotherapy was initiated; however, the patient's clinical condition deteriorated and resulted in death. Pathological examination demonstrated small round blue tumor cells forming Homer-Wright pseudorosettes in both adrenal glands, the liver and the lungs. Immunohistochemical staining showed tumor cells positive for CD56, chromogranin, and synaptophysin, with a Ki-67 proliferation index of approximately 35–40%. Metastatic involvement of the lungs was also identified.

Conclusion: Conclusion: This case highlights a rare presentation of congenital bilateral adrenal neuroblastoma with extensive metastases to the liver and lungs. Massive hepatomegaly may cause respiratory compromise and predispose to severe complications, including infection and multi-organ failure. Early recognition of congenital neuroblastoma is essential for appropriate diagnosis and management.

Abstract 277

Progress on the treatment of hepatoblastoma in children

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Purpose: This review aims to comprehensively review the epidemiological characteristics, pathogenesis, clinical manifestations, diagnostic methods and treatment strategies of hepatoblastoma, in order to provide more detailed reference for clinicians, optimize the treatment plan of patients, and improve the cure rate and quality of life of patients.

Methods: Through a systematic review of high-quality research literature on hepatoblastoma at home and abroad in recent years, including clinical trials, cohort studies, case-control studies, etc., key information was extracted and comprehensively analyzed. At the same time, a comprehensive discussion was formed by combining clinical practice experience and the latest research results.

Results: Hepatoblastoma is more common in infants and young children, and its pathogenesis is related to many genetic and environmental factors. Early diagnosis depends on imaging and pathological examination, and treatment includes surgical resection, chemotherapy and radiotherapy. In recent years, with the promotion of multidisciplinary comprehensive treatment model, the prognosis of patients has been significantly improved.

Conclusion: The comprehensive treatment of hepatoblastoma should be individualized, and reasonable treatment should be selected according to the factors such as age, disease stage and genetic characteristics of patients. In the future, with the development of molecular biology and precision medicine technology, the diagnosis and treatment level of hepatoblastoma is expected to be further improved.

Abstract 278

Clinical features and prognostic analysis of 51 children with rhabdomyosarcoma

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Purpose: To explore clinical characteristics and prognostic factors in children with rhabdomyosarcoma.

Methods: A retrospective analysis was conducted on clinical data of 51 rhabdomyosarcoma (RMS) patients admitted to Children's Hospital of Nanjing Medical University from June 2019 to August 2025. Patients were grouped according to factors including gender, age, tumor origin site, tumor size, clinical stage grouping, risk classification, pathological type, molecular biology, and chemotherapy, calculate the progression-free survival (PFS) and overall survival (OS) of the pediatric patients, and analyze the influencing factors of prognosis.

Results: Among the 51 RMS patients, 27 were males and 24 were females. The median age at onset was 57 (range: 2~164) months. Pathological types: 28 cases of embryonal rhabdomyosarcoma, 21 cases of alveolar rhabdomyosarcoma, and 2

cases of spindle cell rhabdomyosarcoma. There were 6 cases in the low-risk group, 20 cases in the intermediate-risk group, and 25 cases in the high-risk group. All children in the low-risk group achieved complete remission and long-term survival; 3 cases died in the intermediate-risk group (3/20); 5 cases died in the high-risk group (5/25). The 2-year overall survival (OS) rate of the 51 children was 82±6%, and the 2-year progression-free survival (PFS) rate was 77±7%. The 2-year OS and PFS rates of the low-risk group were both 100%; those of the intermediate-risk group were 88±8% and 82±9%, respectively; and those of the high-risk group were 71±11% and 65±11%, respectively. Survival analysis of children in the high-risk group indicated that gender (P=0.03), presence of distant metastasis (P=0.003), pathological type (P 0.001), and presence of recurrence (P 0.001) were factors associated with survival rate.

Conclusion: The retrospective study of our center showed that the overall 2-year OS and PFS of RMS were improved compared with historical data. Gender, distant metastasis, pathological type and recurrence status were prognostic factors for RMS in the high-risk group.

Abstract 279

Clinical features of pediatric malignant solid tumor patients with germline mutations in predisposition genes

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Purpose: Germline variants linked to tumor predisposition are associated with pediatric malignant solid tumors. Efficiently and precisely identifying eligible patients for testing can facilitate clinical management strategies and reduce social costs. Objective To characterize the clinical features of children with malignant solid tumors harboring germline variants associated with tumor predisposition and to inform strategies for genetic screening and tumor surveillance.

Methods: Children with malignant solid tumors and germline variants related to tumor susceptibility who were treated at Beijing Children's Hospital between July 1, 2017 and March 1, 2025 were retrospectively analyzed. Clinical characteristics and genetic testing results were collected.

Results: Fifty-one patients were included (male-to-female ratio, 1.4:1). The median age at tumor onset was 4 years (range, 0 to 17 years), and 68.6% were of preschool age. A family history of cancer was present in 29.8% (14/47), and 11.8% (6/51) exhibited distinctive physical features. Pathogenic or likely pathogenic germline variants were identified in TP53 (n = 17), DICER1 (n = 12), and 21 additional genes. Twenty tumor types were observed, most commonly rhabdomyosarcoma (16/51, 31.4%) and pleuropulmonary blastoma (7/51, 13.7%). Among patients with TP53 variants, the predominant tumor types were rhabdomyosarcoma (35.3%), choroid plexus carcinoma (23.5%), and adrenocortical carcinoma (17.6%). In patients with DICER1 variants, pleuropulmonary blastoma was the most frequent tumor (58.3%). Two patients carried more than one pathogenic or likely pathogenic germline variant. During follow-up, four patients developed two tumors either synchronously or metachronously, with intervals ranging from 0 to 75 months.

Conclusion: Early onset, a family history of cancer, distinctive physical features, or multiple primary tumors in children with solid tumors are indicators of an underlying tumor predisposition syndrome. Germline genetic testing and genetic counseling for affected families should therefore be routinely considered.

Abstract 280

A United Fight for a Rare Challenge: Multimodal, Collaborative Management of DSRCT

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Purpose: Desmoplastic Small Round Cell Tumour (DSRCT) is a rare and highly aggressive malignancy affecting adolescents and young adults. Prognosis remains poor as it often presents with extensive peritoneal involvement. We report the first case of DSRCT in our centre and highlight the importance of multimodal, collaborative management.

Methods: Case presentation

Results: A 17-year-old female presented with progressive abdominal distension and right hypochondrial pain for four months. Imaging revealed a right upper quadrant mass likely arising from the right liver lobe, three liver nodules, a pelvic mass, multiple peritoneal nodules, and metastases to the ovaries and right proximal humerus. Biopsy confirmed EWS-WT1 fusion positive DSRCT. The peritoneal cancer index (PCI) at diagnosis was 9. After fertility preservation, she was treated with the Ewings protocol (VIDE 3-weekly), and responded well after two cycles, with PCI decreasing to 8 and no extra-abdominal metastases. Following literature review and multidisciplinary tumour board discussion, a decision was made to proceed cytoreductive surgery (CRS) and hyperthermic intraperitoneal chemotherapy (HIPEC). As this was the first case in our centre, planning meetings and operating theatre "dry runs" were conducted with all specialties involved, the HIPEC machine vendor and an adult sarcoma team experienced in performing HIPEC. The patient underwent CRS involving non-anatomical right hemihepatectomy, excision of pelvic and omental masses, and peritonectomy, achieving completeness of cytoreduction score 0 (CC-0), followed by HIPEC with cisplatin at 41°C for 90 minutes. Nine days later, radiofrequency ablation of three liver nodules achieved gross complete remission. The postoperative course was uneventful. She completed systemic chemotherapy and is planned for whole abdomen radiotherapy with temozolamide as radiosensitiser, followed by maintenance chemotherapy.

Conclusion: DSRCT poses significant therapeutic challenges due to aggressive biology and diffuse peritoneal dissemination. CRS with HIPEC may achieve locoregional disease control in selected patients. This case highlights the importance of careful patient selection and strong multidisciplinary collaboration.

Abstract 281

Clinical and Prognostic Features in Pediatric Bladder/Prostate Rhabdomyosarcoma: A Retrospective Cohort Analysis

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Purpose: To retrospectively analyze the clinical characteristics, treatment outcomes, and adverse prognostic factors in a single-center cohort of children with bladder/prostate rhabdomyosarcoma (BP-RMS), providing evidence for optimizing treatment strategies.

Methods: Children diagnosed with primary BP-RMS at the Department of Oncology, Hospital from May 2012 to December 2021 were enrolled. A multidisciplinary treatment model (surgery combined with chemotherapy and radiotherapy) was applied, with follow-up ending on December 31, 2024. Clinical data including gender, age at diagnosis, metastasis status, chemotherapy response, resection extent, and radiotherapy were analyzed as prognostic factors for event-free survival (EFS) and overall survival (OS). Recurrence rates were compared using χ^2 tests, and univariate analysis was performed for adverse

prognostic factors.

Results: A total of 32 patients were included (22 males, 10 females), with a median diagnosis age of 35 months (range: 5–137 months). Histologically, 29 (90.6%) were embryonal type and 3 (9.4%) alveolar type. IRS staging: III (84.4%, 27/32) and IV (15.6%, 5/32); TNM staging: 2 (43.8%, 14/32), 3 (40.6%, 13/32), and 4 (15.6%, 5/32). All patients received chemotherapy, 23 (71.9%) underwent radiotherapy, and 23 (71.9%) underwent surgery: R0/R1 resection (25%, 8/32) and R2 resection (46.9%, 15/32); 9 (28.1%) did not undergo surgery. Median follow-up was 72.5 months (range: 12–152 months). Disease progression occurred in 9 cases (28.1%, median time: 13 months) and recurrence in 7 (18.8%, median time: 16 months). The 3-year EFS and OS for the entire cohort were 53.1%±8.8% and 68.8%±8.2%, respectively. For 27 non-metastatic BP-RMS patients, 3-year EFS and OS were 59.35%±9.5% and 70.4%±9.5%. The R0/R1 resection + radiotherapy group achieved 100% 3-year EFS, significantly higher than the R2 resection + radiotherapy group (61.5%±13.5%), though without statistical significance (χ^2=2.768, P=0.096). Among induction chemotherapy-responsive patients, R2 resection (14 cases) and non-surgical (5 cases) showed no significant difference in progression/recurrence rate (42.9% vs. 60%, χ^2=0.434, P=0.51) or survival (35.7% vs. 40%, χ^2=0.029, P=0.865). The 3-year EFS of chemotherapy-responsive vs. non-responsive groups was 61.9%±10.6% vs. 30%±14.5% (χ^2=3.132, P=0.077), while 3-year OS was significantly higher in the responsive group (81.0%±8.6% vs. 40%±15.5%, χ^2=6.903, P=0.009). Among 10 chemotherapy-nonresponsive (PD/SD) patients, only 1 (14.3%) achieved PR after intensified chemotherapy, with 5 deaths. Radiotherapy significantly improved 3-year EFS (73.9%±9.2% vs. 0%, χ^2=29.612, P<0.001) and OS (87.0%±0.7% vs. 25.0%±15.3%, χ^2=14.881, P<0.001). T1 localized tumors showed higher 3-year EFS than T2 tumors (88.9%±10.5% vs. 39.1%±10.2%, χ^2=5.803, P=0.016), but no OS difference.

Conclusion: R0/R1 resection combined with radiotherapy improves local control and survival in pediatric BP-RMS. For chemotherapy-responsive patients unable to achieve R0/R1 resection, R2 resection does not reduce recurrence risk. Intensified chemotherapy offers limited benefit for nonresponsive patients, warranting early treatment strategy adjustments.

Abstract 282

EXTRARENAL RETROPERITONEAL WILMS TUMOR IN A CHILD WITH HIGH TUMOR MUTATIONAL BURDEN: A RARE CASE WITH COMPREHENSIVE MOLECULAR PROFILING

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Purpose: Extrarenal Wilms tumor (ERWT) is an exceptionally rare embryonal neoplasm comprising less than 1% of all Wilms tumors. Diagnosis is challenging due to morphological overlap with other retroperitoneal tumors of childhood. Comprehensive molecular profiling of ERWT using next-generation sequencing (NGS) has rarely been reported, and no molecular data exist from Mongolia.

Methods: A 3-year-11-month-old Mongolian girl presented with a progressive abdominal mass. Surgical resection was performed at the National Children's Medical Center, Ulaanbaatar. Histopathological and immunohistochemical analyses were performed at the National Center for Pathology, Mongolia. As molecular genetic testing is not available in Mongolia, the FFPE tissue block was sent to Xintu Diagnostics Laboratory (Hunan, China) for comprehensive NGS using the Xintu DNA PanCancer Panel v1.1 covering 620 genes.

Results: A 12.5 x 10 x 7.5 cm retroperitoneal mass, completely separate from both kidneys, was resected (yp Stage II). Histopathology demonstrated blastemal-predominant nephroblastoma. Immunohistochemistry confirmed WT1(+), PAX8(+), Vimentin(+), CD56(+), Ki-67 greater than 10%, Synaptophysin(-), excluding neuroblastoma, rhabdomyosarcoma, and Ewing sarcoma. NGS identified 43 somatic mutations. No pathogenic mutations were detected in canonical Wilms tumor driver genes (WT1, CTNNB1, DROSHA, WTX/AMER1, DICER1, MYCN). Tumor mutational burden (TMB) was high at 14.02 mut/Mb, meeting the FDA threshold for pembrolizumab eligibility in TMB-high solid tumors. Microsatellite status was stable (MSS). No pathogenic germline mutations were identified. Dominant somatic alterations involved epigenetic and chromatin remodeling pathways (ZFHX3, ARID1B, KDM6A, SMAD2).

Conclusion: This represents the first molecularly characterized ERWT from Mongolia. Absent canonical Wilms driver mutations and a predominance of epigenetic pathway alterations suggest a distinct molecular pathogenesis for the extrarenal variant. The TMB-high finding — identified through international molecular collaboration — documents a potential immunotherapy option in the relapse setting. This case highlights the clinical value of overseas NGS referral for rare pediatric tumors in resource-limited settings.

Abstract 283

Limited Survival Benefit of Topotecan/Vincristine/Doxorubicin(TVD) Salvage Chemotherapy in Primary Refractory High-Risk Neuroblastoma: A Single-Institution Experience

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Purpose: Management of high-risk neuroblastoma(HR-NB) in low-resource settings remains challenging due to limited access to anti-GD2 immunotherapy. Primary refractory disease further complicates treatment, as resistance to first-line chemotherapy is strongly associated with inferior outcomes. We report single-institution outcomes of primary refractory neuroblastoma treated with topotecan–vincristine–doxorubicin(TVD) salvage chemotherapy.

Methods: Primary refractory HR-NB patients treated between July 2018 and July 2023 were included. Primary refractory disease was defined according to SIOPEN criteria as persistence of ≥3 metastatic sites on ^131I-MIBG imaging and/or bone marrow involvement following rCOJEC induction chemotherapy. Patients received two cycles of salvage TVD followed by response assessment. Those achieving metastatic complete response(mCR) or very good partial response(mVGPR) proceeded to surgery, autologous stem cell transplantation(ASCT) with busulfan–melphalan conditioning, radiotherapy to the primary and/or residual metastatic sites, and 13-cis retinoic acid maintenance. Anti-GD2 immunotherapy with Dinutuximab-beta was administered when accessible. Statistical analysis was performed using IBM-SPSS Statistics version 25.

Results: Among 174 HR-NB patients, 56(32.2%) had primary refractory disease; 27(48.2%) who proceeded with curative-intent therapy using TVD were included. Median age was 54 months with a male-to-female ratio of 3:1. MYCN amplification was present in 3(11.1%), absent in 15(55.5%), and unavailable in 9(33.3%). Following two cycles of TVD, objective response(CR+VGPR) was observed in 14 patients(51.9%). Gross total resection was achieved in 13(92.8%). All patients subsequently underwent ASCT, radiotherapy, and 13-cis retinoic acid maintenance; three additionally received dinutuximab-beta. At a median follow-up of 30.9 months(23.7–38.1), 3-year event-free survival was 6.5±0.5% and overall survival was 25.9±1.2%. All patients receiving anti-GD2 maintenance subsequently relapsed.

Conclusion: Salvage chemotherapy with topotecan, vincristine, and doxorubicin provides limited survival benefit in primary refractory HR-NB. Persistently poor outcomes in LMIC settings highlight the urgent need for improved access to effective therapies, particularly incorporation of anti-GD2 immunotherapy into frontline treatment strategies

Abstract 284

Summary of a Multicenter Clinical Case Series on MS Stage Neuroblastoma

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Purpose: MS stage neuroblastoma exhibits unique biological behavior and a favorable prognosis. This study retrospectively summarizes its clinical characteristics and prognostic outcomes to provide references for future treatment strategies.

Methods: This study incorporated data from multiple pediatric oncology centers. A retrospective analysis was conducted on 47 cases of MS stage neuroblastoma

diagnosed between January 2015 and December 2025. Patients were grouped and compared based on hepatic metastasis status, MYCN gene amplification status, pathological subtype, and prognostic outcome. The Kaplan-Meier method and Cox regression analysis were employed to identify prognostic factors.

Results: A total of 47 children with MS stage neuroblastoma were enrolled, including 31 males (66.0%). The median age was 2.8 months (range: 0–15 months). The most common primary site was the adrenal gland (68.1%). The incidence of hepatic, skin, and bone marrow metastases were 87.2%, 12.8%, and 8.5%, respectively. Regarding treatment, 86.7% of the patients underwent surgery, and 81.0% received chemotherapy. The median follow-up time was 36.0 months. During follow-up, 5 patients (11.1%) died, and 40 patients (88.9%) survived. The MYCN gene amplification rate was 20.9%. Inter-group comparisons showed that the group without hepatic metastasis had lower ferritin levels and longer survival times ($P < 0.05$); the MYCN amplification group had significantly higher ferritin and NSE levels and shorter survival times ($P < 0.05$); different pathological subtype groups showed differences in skin metastasis, hepatic metastasis, and chromosome 11q status ($P < 0.05$); the LDH level in the deceased group was significantly higher than that in the survival group ($P = 0.005$). Univariate Cox regression analysis identified MYCN gene amplification (HR = 10.802), elevated LDH (HR = 1.001), and elevated ferritin (HR = 1.002) as independent risk factors for mortality in children with MS stage disease (all $P < 0.05$).

Conclusion: MYCN amplification, elevated LDH, and elevated ferritin are independent risk factors affecting the prognosis of children with MS stage neuroblastoma. This confirms significant heterogeneity within the MS stage category, suggesting that intensified treatment regimens are necessary for patients presenting with these high-risk factors.

Abstract 285

BRD4 INHIBITION TARGETS THE MYCN-GLUTAMINE METABOLISM AXIS TO SUPPRESS THE MALIGNANT PROGRESSION OF NEUROBLASTOMA

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Purpose: Neuroblastoma (NB) is an embryonal tumor originating from the malignant transformation of neural crest cells (NCCs), and its profound clinical heterogeneity is closely linked to glutamine metabolism. BRD4, an epigenetic “reader” protein of the BET (bromodomain and extraterminal) family, is significantly overexpressed in NB, particularly in MYCN-amplified subtypes. By recognizing acetylated histones (e.g., H3K27ac) enriched at super-enhancer (SE) regions, BRD4 drives the transcription of critical oncogenes like MYCN and NCC stemness-related genes. Furthermore, BRD4 participates in an “acetylation-methylation” switch at the histone H3K27 site, recognizing newly acquired H3K27ac modifications to amplify transcriptional activation signals and synergistically drive tumor malignant progression. This study aims to elucidate the mechanism by which BRD4 inhibitors target the MYCN-glutamine metabolism axis to regulate NCC developmental plasticity and intervene in NB progression.

Methods: A comprehensive multi-level approach was employed to investigate the targeted interventions: (1) At the molecular level, the transcriptional activation mechanisms of MYCN regulated by BRD4 and its key functional domains were verified. (2) At the cellular level, the dynamic reprogramming effects of BRD4 inhibition on the glutamine metabolism axis and NADPH redox homeostasis were evaluated in MYCN-amplified NB cell lines. (3) At the animal level, NB xenograft mouse models were established to validate the in vivo anti-tumor efficacy of BRD4 inhibitors targeting the MYCN-glutamine metabolism axis.

Results: Pharmacological inhibition of BRD4 effectively blocked the compensatory transcriptional mechanisms driven by the H3K27 modification switch. BRD4 inhibitors significantly downregulated MYCN protein expression in MYCN-amplified NB cells. This targeted downregulation led to a profound suppression of tumor-specific glutamine metabolism and disrupted NADPH redox homeostasis, impairing the developmental plasticity of NCCs. Consistent with in vitro findings, in vivo xenograft models demonstrated that BRD4 inhibition remarkably attenuated tumor growth by disrupting the MYCN-glutamine metabolic dependency, exhibiting robust anti-tumor effects.

Conclusion: BRD4 acts as a pivotal epigenetic driver in NB by synergizing transcriptional regulation with metabolic reprogramming. Inhibiting BRD4 effectively uncouples the MYCN-glutamine metabolism axis, disrupting both metabolic and redox homeostasis essential for tumor survival. These findings elucidate the critical role of BRD4 in NB malignant progression and provide a compelling mechanistic rationale for utilizing BRD4 inhibitors as a promising epigenetic-metabolic therapeutic strategy for MYCN-amplified Neuroblastoma.

Abstract 286

Cystic bilateral Wilms tumor - a description of rare case.

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Purpose: Wilms tumor (WT), or nephroblastoma, is the most common renal cancer in the pediatric age group. It is thought to develop from persistent metanephric tissue or nephrogenic rests. These cells are found in up to 100% of cases of bilateral Wilms tumor.

Methods: We report a 11-month-old boy presented with unusual type of bilateral WT with a predominance of the multilocular cystic mass, who was treated at Dmitry Rogachev NMRC.

Results: A 11-month-old boy presented with an abdominal mass, lack of weight gain and decrease in appetite. Clinical condition was mostly stable with the exception of arterial hypertension. From MRI and CT, differential diagnosis was between bilateral WT and nephroblastomatosis due to cystic components. There was no evidence of metastases. According to The UMBRELLA SIOP–RTSG 2016 Protocol, due to the age and radiological characteristics, neoadjuvant chemotherapy was started (AV 6 weeks). However, after 3 weeks of AV, an increase in the cystic components was detected in both kidney but solid parts of the tumors in dynamic decreased. The patient subsequently underwent a simultaneous bilateral radical nephron-sparing surgery. Histopathological examination revealed WT with epithelial and stromal components in septs, cysts were found in all areas of the tumors. The patient was diagnosed with WT, mixed type, stage II (due to renal sinus invasion), R0-resection on both side. Patient underwent 27 weeks of adjuvant chemotherapy (Regimen AV-2) in good clinical condition and with complete response. The follow-up period was 35 months.

Conclusion: The rare cystic form of bilateral WT was presented in the case. Preoperative chemotherapy may be administered in the presence of a solid component, but due to a lack of positive dynamics from cysts the surgical treatment at an earlier time ought to be considered.

Abstract 287

Clinical Characteristics, Molecular Profiling, and Treatment Outcomes of Pediatric Non-Rhabdomyosarcoma Soft Tissue Sarcomas: A Single-Center Retrospective Analysis of 12 Cases with Exploration of Hematopoietic Stem Cell Transplantation

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Purpose: Pediatric non-rhabdomyosarcoma soft tissue sarcomas (NRSTS) represent a heterogeneous group of aggressive malignancies lacking standardized treatment protocols. With advances in molecular diagnostics, precise classification and tailored therapies are pivotal for improving outcomes. This study aimed to characterize the clinical features, molecular genetic landscape, and efficacy of multidisciplinary treatment (MDT) in children with NRSTS, and to evaluate the potential role of peripheral blood stem cell transplantation (PBSCT) in relapsed/refractory cases.

Methods: We retrospectively analyzed 12 pediatric patients diagnosed with NRSTS at Sun Yat-sen Memorial Hospital between July 2017 and July 2022. Data on clinicopathological characteristics, next-generation sequencing (NGS) results, and treatment responses were collected. All patients received multimodal therapy including surgery, chemotherapy, and/or radiotherapy. Clinical data, tumor genomic profiling results, treatment responses, and follow-up outcomes were collected. Prognostic factors were analyzed using logistic regression. Event-free survival (EFS) was estimated by Kaplan–Meier method.

Results: The cohort included 12 patients (3 males, 9 females) with a median age of 8.6 years (range: 1.1–13.1). The head and neck was the primary site in 66.7% (8/12) of cases. Most patients presented with stage III disease (75%, 9/12) and tumors >5 cm (58.3%, 7/12). Histological subtypes were diverse, with extraosseous Ewing sarcoma being the most common (4/12). Molecular profiling (n=9) revealed EWSR1-FLI1 fusions in 3 cases, ETV6-NTRK3 fusion in infantile fibrosarcoma, TFE3 rearrangement in alveolar soft part sarcoma, and SMARCA4 loss in one undifferentiated case. Regarding treatment, 11 patients (91.7%) underwent radical surgery (R0 resection in 8), and 10 received radiotherapy. The overall response rate (ORR) to initial therapy was 91.7% (10 complete responses, 1 partial response). With a median follow-up of 21.6 months (range: 2–40), the 3-year EFS was 57.1% (95% CI: 28.5%–85.8%). The relapse rate was 50% (6/12), with a median time to relapse of 12 months. Multivariate analysis identified age >9 years ($p<0.05$) and incomplete surgical resection ($p<0.05$) as independent risk factors for poor prognosis. Notably, two relapsed patients underwent haploidentical PBSCT; both achieved successful engraftment without transplant-related mortality and remain alive at 36 and 40 months post-transplant, respectively.

Conclusion: Pediatric NRSTS exhibits significant heterogeneity, underscoring the critical value of molecular testing for precise diagnosis. While MDT yields high initial response rates, older age and positive surgical margins are associated with a heightened risk of relapse. Allogeneic PBSCT emerges as a promising salvage strategy for selected relapsed/refractory NRSTS cases, warranting further investigation in larger cohorts.

Abstract 288

Locally advanced and destructive desmoid fibromatosis of the Th11-L1 vertebra. Description of the clinical case.

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Desmoid tumors (desmoid, desmoid fibromatosis, aggressive fibromatosis, DF) are a group of mesenchymal tumors with uncertain or low malignancy potential. They lack metastatic potential but are characterized by aggressive growth and a high recurrence rate. Radical surgery has traditionally been the primary treatment method, but combination of drug therapy and radiation therapy are currently the main methods for tumor reduction.

Purpose: Desmoid tumors (desmoid, desmoid fibromatosis, aggressive fibromatosis, DF) are a group of mesenchymal tumors with uncertain or low malignancy potential. They lack metastatic potential but are characterized by aggressive growth and a high recurrence rate. Radical surgery has traditionally been the primary treatment method, but combination of drug therapy and radiation therapy are currently the main methods for tumor reduction.

Methods: We describe a clinical case of desmoid fibromatosis of the Th11-L1 vertebra with a locally advanced, destructive growth in a 10-year-old female patient. At admission, the patient exhibited severe pain syndrome, which was relieved by the using of a narcotic analgesic—1% morphine hydrochloride solution. Neurological deficits were also noted, including "sock-like" paresthesia, decreased muscle strength in the lower extremities (up to 4 points proximally and up to 3 points distally), and pelvic dysfunction (urinary dysfunction).

Results: For vital reasons, pending the histological report, a decision was made to initiate a specific treatment with sarcoma-targeted regimen VDC (vincristine, doxorubicin, cyclophosphamide). After the first course of chemotherapy, on day 10, the child experienced pain relief and resolution of pelvic disorders. After two courses of chemotherapy, MRI showed positive dynamics, with a 45% tumor reduction. Radiation therapy to the primary site is planned after 6 courses. An FMI study (Foundation Medicine Inc.) identified the APC gene, which predisposes to sporadic cases of DF and increases the risk of recurrence.

Conclusion: There are currently no uniform treatment protocols for patients with desmoid fibromatosis. Despite advances in surgical treatment and drug therapy, desmoid tumors remain a urgent problem in pediatric oncology. A personalized approach to therapy is needed to improve treatment outcomes and quality of life. Low-dose chemotherapy regimens are often used in the treatment of desmoid fibromatosis, but because of the extremely aggressive behavior of tumor at manifestation, typical of high-grade sarcomas, more aggressive treatment regimens are also possible to used.

Abstract 289

Mechanisms of synergistic lethality in combined epigenetic therapy for neuroblastoma: EZH2 derepression activates QRICH2 to drive differentiation and metabolic remodeling

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Purpose: Phenotypic plasticity constitutes the fundamental driver of therapeutic resistance and relapse in high-risk neuroblastoma (NB). While epigenetic vulnerabilities are heavily implicated in NB progression, single-agent interventions frequently encounter adaptive resistance. This study aims to elucidate the mechanisms underlying the robust synergistic lethality observed upon combined epigenetic inhibition of BRD4 and EZH2 in MYCN-amplified NB, specifically focusing on how the novel molecular hub QRICH2 orchestrates the crosstalk between epigenetic remodeling and mitochondrial metabolic decompensation.

Methods: This project integrates label-free metabolic imaging and multi-omics profiling (including CUT&Tag and transcriptomics) to dissect epigenetic and metabolic alterations. The mechanistic hypotheses and synergistic anti-tumor efficacy of the dual BRD4/EZH2 targeted strategy will be systematically evaluated across a physiologically relevant hierarchy of preclinical models, including in vitro NB cell lines, human sympathetic ganglion organoids (hSGO-NCC), malignant transformation models, and in vivo cell-line-derived xenograft (CDX) models. Dynamic monitoring of the mitochondrial electron transport chain (ETC) and oxidative stress parameters will be conducted to validate the metabolic collapse.

Results: Preliminary data demonstrate that combined inhibition of BRD4 and EZH2 exerts a profound synergistic lethal effect on MYCN-amplified NB cells. Mechanistically, label-free imaging and multi-omics reveal that EZH2 inhibition abrogates the repressive H3K27me3 modification at the QRICH2promoter, leading to its robust derepression. The activated QRICH2 subsequently drives a transition toward a highly proliferative and metabolically demanding adrenergic (ADR) phenotype by mediating the AMPK-SLC25A4 axis. Concurrently, BRD4 inhibition effectively antagonizes the MYCN-driven energy supply network. This simultaneous energy deprivation and forced high-metabolic demand precipitate a severe energetic supply-demand mismatch, ultimately resulting in ETC structural impairment, lethal reactive oxygen species (ROS) accumulation, and apoptosis.

Conclusion: QRICH2 acts as a critical mechanistic nexus bridging epigenetic derepression and mitochondrial metabolic rewiring. The synergistic strategy of dual BRD4 and EZH2 inhibition exploits a lethal “epigenetic-metabolic conflict,” selectively driving MYCN-amplified NB cells toward mitochondrial decompensation and apoptosis. Elucidating this regulatory network provides a compelling, mechanistically validated therapeutic paradigm to overcome drug resistance in high-risk NB.

Abstract 290

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Purpose: Osteosarcoma (OS) is the most common primary malignant bone tumor in children and adolescents and is characterized by malignant osteoid production and a high risk of metastasis. The 5-year survival rate for patients with localized disease is approximately 67%, whereas survival remains around 20% in patients with metastatic disease. In Vietnam, reported outcomes for osteosarcoma range from 13% to 63%

Methods: This retrospective study was conducted on pediatric patients diagnosed with osteosarcoma at Ho Chi Minh City Children's Hospital from January 2020 to May 2025. Information on epidemiological characteristics, tumor characteristics, treatment methods, and treatment outcomes was collected from medical records. Overall survival (OS) and event-free survival (EFS) were analyzed, and prognostic factors were assessed using Cox regression.

Results: A total of 34 patients were included. The median age at diagnosis was 10 years, with a male-to-female ratio of 1.5:1. According to AJCC 8th staging, 47.1% of patients presented with metastatic disease at diagnosis. Most patients (88.2%) were classified as high- or very-high-risk. All patients received chemotherapy,

mainly the MAP regimen (88.2%), and 79.4% underwent surgical resection, including 51.5% amputation and 27.3% limb-salvage surgery. The estimated OS at 12 and 24 months was 71.6% and 42.8%, respectively, with a median OS of 23.3 months. The EFS at 12 and 24 months was 58.8% and 42.8%, respectively. Metastasis at diagnosis and severe treatment-related complications were associated with poorer survival, whereas surgical treatment significantly improved outcomes.

Conclusion: Pediatric osteosarcoma patients at our center often present at an advanced stage and with a late diagnosis. Multimodal treatment is feasible; however, early detection and improved referral systems are essential to improve treatment outcomes in Vietnam.

Abstract 291

CLINICAL PROFILE AND OUTCOME OF CHILDREN WITH WILMS TUMOR TREATED ACCORDING TO THE INTERNATIONAL SOCIETY OF PEDIATRIC ONCOLOGY (SIOP) PROTOCOL IN A TERTIARY PUBLIC HOSPITAL

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Purpose: Wilms tumor (WT) is the most common pediatric renal malignancy, represen ng approximately 90% of childhood kidney cancers. While survival exceeds 90% in high-income countries, outcomes in low- and middle-income countries (LMICs) are hampered by delayed diagnosis and treatment abandonment. This study aimed to evaluate the clinical profile and survival of WT patients treated under the International Society of Pediatric Oncology (SIOP) protocol.

Methods: A retrospec ve cohort study included children diagnosed with WT between 2015 2024 and managed using SIOP protocol. Overall survival (OS) and event-free survival (EFS) were esmated using Kaplan–Meier analysis.

Results: A total of 63 patients were analyzed with mean age of 4.7 years and male predominance (61.9%). Most presented with abdominal mass (84.1%) and 49.2% presented with metastatic disease, primarily in the lungs (53.85%). All patients began preoperative chemotherapy but only 79.4% underwent surgery. The treatment completion rate was 55.6%, with a high abandonment rate of 44.4%. Histology was intermediate-risk in 64%, high-risk in 32%, and low-risk in 4%. The 1-year and 3-year OS were 53.9% and 22.8%, respectively; 3-year EFS was 30.4%. The 3-year OS for those who completed treatment was 62.9%, compared to 0% for those who abandoned ($p < 0.001$). Paradoxically, treatment delays were associated with higher survival ($p < 0.001$), likely reflecting sustained engagement with the healthcare system versus early abandonment. Adjuvant radiotherapy, though used in only 11.1% of cases, resulted in 100% survival.

Conclusion: Implementa on of the SIOP protocol provided a standard framework, but survival remains subopmal due to late-stage presenta on and high abandonment rates. These findings underscore that clinical protocols must be paired with aggressive social support and early detec on programs to bridge the survival gap in resource-limited settings.

Abstract 292

Survival Outcomes in Bilateral Wilms Tumor with High-Risk or Unfavorable Histology: A Multicenter Cohort Study from China

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Purpose: The presence of high-risk histology (HRH) or unfavorable histology (UH) in bilateral Wilms tumor (BWT) is extremely rare, and the oncological outcomes following nephron-sparing surgery (NSS) in this population remain unclear. This multicenter study aims to characterize clinical features and survival outcomes.

Methods: BWT patients from Chinese multicenter collaborative groups were enrolled. All underwent at least unilateral NSS and received standardized treatment per SIOP or COG protocols. Clinical data were collected via case report forms, with central pathological review of all high-risk components or UH. Systematic follow-up was conducted for patients with HRH/UH identified in prior retrospective studies to analyze treatment modalities, recurrence patterns, and survival.

Results: Among 167 BWT patients, central review identified 16 (9.6%) with HRH/UH, involving 18 affected kidneys. Procedures included 15 NSS and 3 radical nephrectomies (RN). Two patients exhibited anaplasia (focal in one, diffuse in one), with intraoperative tumor rupture in the focal case. Fourteen patients (16 kidneys) showed chemotherapy-predominant blastemal subtype (high-risk pathology). No patient underwent salvage RN in the short term specifically due to HRH or UH. At a median follow-up of 43 months, 3 deaths occurred (all from progressive lung metastases). Eight patients experienced recurrence, metastasis, or progression: One patient with diffuse anaplasia experienced progression of residual nephroblastomatosis postoperatively and achieved event-free survival after repeat NSS plus multimodal therapy. One patient with focal anaplasia and intraoperative rupture underwent RN, developed local ecurrence, and achieved event-free survival after surgery and comprehensive treatment. The remaining six presented with new or progressive lung metastases; three achieved event-free survival after multimodal therapy, while three died. Survival analysis revealed no significant difference in overall survival (OS) between HRH/UH and non-HRH/UH groups (Fig A), but event-free survival (EFS) differed significantly (Fig B). Within the HRH/UH group, NSS did not significantly impact OS or EFS (Fig C, D).

Conclusion: In Chinese BWT patients, UH incidence is 1.1%, with high-risk pathology predominantly comprising post-chemotherapy blastemal subtype. Pulmonary metastasis is the predominant recurrence pattern. Under standardized treatment and meticulous surgical technique, NSS does not compromise oncological outcomes in HRH/UH patients.

Abstract 293

Beyond the Brain and Kidneys: A Rare Case of Malignant Rhabdoid Tumor of the Bladder

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Purpose: Malignant rhabdoid tumors (MRT) are highly aggressive pediatric malignancies typically found in the kidneys or central nervous system (CNS). Extrarenal, non-CNS MRTs are exceedingly rare, with an annual incidence of approximately 0.6 per million children. These tumors are characterized by alterations in SMARCB1 gene and typically demonstrate loss of nuclear INI1 expression on immunohistochemistry. We present a rare case of primary MRT of the urinary bladder emphasizing the diagnostic challenges and necessity of specialized immunohistochemistry

Methods: An 8-year-old, Filipino, male presented with a one-month history of gross hematuria. Physical examination revealed a 9×9 cm firm mass in the mid-hypogastric region and bilateral inguinal lymphadenopathies. Imaging showed a large, fungating intraluminal soft-tissue mass from the left lateral wall of the urinary bladder with signs of necrosis. Initial histopathology showed atypical round to polygonal cells suggestive of rhabdomyosarcoma. Definitive immunohistochemistry revealed loss of nuclear INI1 expression alongside positivity for WT1, CD99, and vimentin, confirming a diagnosis of MRT.

Results: The patient was initially treated with a VAC (vincristine, actinomycin, cyclophosphamide) regimen for rhabdomyosarcoma and radiotherapy for local control. Upon confirmation of MRT, therapy was intensified to an ICE (ifosfamide, etoposide, carboplatin) regimen and definitive surgery with radical cystectomy, bilateral pelvic lymph node dissection, and an ileal conduit urinary diversion. Most recent follow-up revealed stable disease. The post-therapeutic course has been complicated solely by recurrent urinary tract infections related to the ileal conduit urinary diversion, which are being managed with therapeutic and prophylactic antibiotics.

Conclusion: Primary MRT of the bladder is exceptionally rare and carries a poor prognosis without aggressive intervention. This case underscores the importance of including MRT in the differentials of pediatric bladder masses and the role of INI1 staining in ensuring correct diagnosis. Reporting this is vital to refining multimodal treatment strategies for this challenging case.

Abstract 294

Transarterial Chemoembolization as a Neoadjuvant Strategy for High-Risk Hepatoblastoma with Rare Bone Marrow Metastasis

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Purpose: Hepatoblastoma typically metastasizes to the lungs. Isolated bone marrow metastasis in the absence of pulmonary involvement is an exceedingly rare presentation. We report the management of a technically unresectable, high-risk case where Transarterial Chemoembolization (TACE) was utilized as a bridge toward definitive therapy.

Methods: A 3-year-old, Filipino, female presented with abdominal mass. Biopsy revealed Hepatoblastoma, epithelial type. Triphasic CT scan showed PRETEXT IV (+Caudate lobe, +Portal vein tumor thrombus, +F multiFocal, +N lymph nodes). Initial staging confirmed bone marrow metastases (+M), uniquely occurring without evidence of lung involvement. The patient underwent chemotherapy using SIOPEL IV Induction with dose-dense Cisplatin and Doxorubicin, followed by interventional oncology consisting of one cycle of TACE with Doxorubicin. This was followed by two additional cycles of pre-operative chemotherapy with Carboplatin and Doxorubicin.

Results: The regimen yielded a dramatic biochemical response, with Alpha-fetoprotein (AFP) levels decreasing from baseline of >80,000 to 200.76 ng/ml. Repeat bone marrow was negative for tumor cells. A post-TACE PET/CT scan demonstrated an interval decrease in the number and size of hepatic lesions. Active metabolic activity was strikingly localized to only two areas: a persistently active nodule in Segment 8 and residual mass in Segment IVA. Skeletal imaging showed diffuse reactive activity but no focal hypermetabolic lesions, indicating metabolic resolution of initial bone marrow metastases. Although the tumor burden was reduced, the development of cavernous transformation and cirrhosis rendered the patient technically unresectable via standard partial hepatectomy and is for liver transplant.

Conclusion: This case underscores the utility of TACE in achieving a profound metabolic response in high-risk hepatoblastoma with rare metastatic sites. The successful downstaging to just two active segments highlights the role of multimodal interventional strategies in bridging technically complex patients to Living Donor Liver Transplantation.

Abstract 295

A study of Dinutuximab β in the treatment of children with recurrent/refractory neuroblastoma

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Purpose: GD2 antibody Datuximab β Approved by the European Medicines Agency in 2017 for immunotherapy of high-risk, recurrent/refractory neuroblastoma for initial treatment; Starting from November 2020, "pilot trials" of advanced medication were conducted in Hainan and Tianjin medical pilot zones. December 2021 Datuximab β Listing in China brings more hope to Chinese patients with neuroblastoma. Based on the experience of medication in Hainan and Tianjin, the clinical application collaboration group of GD2 monoclonal antibody in the treatment of neuroblastoma released the first GD2 antibody Datuximab in 2022 β Expert consensus on the clinical application of treating neuroblastoma. Bringing good news to Chinese patients with neuroblastoma.The safety and efficacy of Dinutuximab β GD2 monoclonal antibody in the treatment of children with relapsed/refractory neuroblastoma NB were evaluated.

Methods: Among the 12 children with NB there were 7 male and 5 female 1 relapsed patients and 11 refractory patients. A total of 57 cycles of dinutuximabβimmunotherapy were completed.

Results: The pain scores of CRIES in this group were ≤3 and the major manifestations were limb or abdominal pain. All 12 children had fever with an average peak of 39 . The rate of grade ≥3 infection was 16.%. One children allergic constitution (who has previously developed Steven Johnson syndrome during transplantation) were diagnosed as severe capillary leakage syndrome. Up to the last follow-up time the prognosis of 8 patients was improved but 4 cases experienced progressive disease,ALLwere intracranial metastases, 3 survived with tumors and 1 died.

Conclusion: Dinutuximab β is well tolerated relapsing/refractory neuroblastoma patients. The short-term efficacy is good, while the long-term efficacy needs to be further observed.

Abstract 296

Analysis of the therapeutic effect of immunoglobulin shock therapy in patients with myelodysplastic neuroblastoma

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Purpose: To investigate the bone marrow recovery of children with recurrent neuroblastoma and low bone marrow proliferation after long-term treatment with immunoglobulin application.

Methods: A retrospective summary was conducted on the basic data of 12 patients with recurrent neuroblastoma treated with intravenous immunoglobulin from September 20, 2024 to December 20, 2024. The reasons for low bone marrow proliferation and the recovery of bone marrow with immunoglobulin were analyzed.

Results: Among the 12 patients with recurrent neuroblastoma, there were 7 males and 5 females; Eight cases underwent autologous stem cell transplantation, while four cases did not undergo autologous stem cell transplantation; There were 3 patients who received 10-20 cycles of chemotherapy in the past, 9 patients who received 21-41 cycles of chemotherapy; All 12 patients received radiotherapy; Five patients had extremely low bone marrow morphology, while four patients had active bone marrow proliferation but no megakaryocytes were observed; Two cases of infiltration of bone marrow tumor cells were observed, and all lineages were inhibited; After immunoglobulin shock therapy, 5 patients with extremely low proliferation showed 4 cases of bone marrow recovery to active proliferation, 1 case showed primitive cell clusters in bone marrow recovery, 4 cases showed megakaryocytes in bone marrow after treatment despite active proliferation, and 2 cases of bone marrow tumor cell infiltration did not recover. No complications occurred.

Conclusion: Immunoglobulin shock therapy is effective for patients with myelodysplastic disorders;

Abstract 297

Unmasking a Rare Pediatric Intranasal Tumor: CRTC1-MAML2 Fusion Confirmation

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Purpose: Mucoepidermoid carcinoma (MEC) is very rare salivary gland carcinoma in children and even more uncommon in the nasopharynx. Because it can mimic more common tumors such as nasopharyngeal carcinoma, accurate diagnosis can be challenging. We saw an 11-year-old Filipino girl with a three-year history of right-sided nasal obstruction and discharge.

Methods: Nasal endoscopy was conducted followed by tissue biopsy for histopathologic evaluation. Initial histology was further characterized using immunohistochemical staining, including p63 and PAX8, and molecular testing for gene fusion analysis to aid in tumor classification. Cross-sectional imaging was obtained to assess the extent of the lesion and evaluate for regional or distant spread. The patient then underwent surgical management through endoscopic excision with right turbinectomy. Postoperative management included adjuvant radiotherapy.

Results: Nasal endoscopy showed a friable intranasal mass. The initial biopsy read as non-keratinizing squamous cell carcinoma, but further tests revealed diffuse p63 positivity, PAX8 negativity, and most importantly, a CRTC1-MAML2 fusion, confirming Mucoepidermoid Carcinoma of the right intranasal region. Imaging showed a localized 2.3 × 2.0 × 4.2 cm mass without distant spread. The patient underwent complete endoscopic excision with right turbinectomy, achieving negative margins, followed by adjuvant radiotherapy (30 fractions, 60 Gy).

Conclusion: This case highlights how a rare diagnosis like MEC can easily be mistaken for more common head and neck tumors in children. Recognizing the CRTC1-MAML2 fusion was key to confirming the diagnosis and tailoring treatment appropriately. For pediatric oncologists, this underlines the value of molecular testing in guiding both diagnosis and management in uncommon pediatric cancers.

Abstract 298

Interventional Effect of Teniposide-Based Chemotherapy Combined with Intrathecal Injection in Neuroblastoma with Central Nervous System Metastasis: A Retrospective Analysis of 43 Cases

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Purpose: With the advancement of systemic treatment regimens for neuroblastoma, the survival rate of high-risk pediatric patients has improved. However, central nervous system metastasis has become a major cause of treatment failure. This study aimed to investigate the interventional efficacy and prognostic impact of teniposide-based chemotherapy combined with intrathecal injection in patients with neuroblastoma complicated by central nervous system metastasis.

Methods: This retrospective analysis enrolled 43 patients with high-risk neuroblastoma accompanied by central nervous system metastasis who were admitted to Beijing Jingdu Children's Hospital from July 2018 to August 2025. All patients received comprehensive treatment consisting of teniposide combined with intrathecal injection.

Results: The median survival time of the 43 patients after diagnosis of central nervous system metastasis was 17.0 months. The 12-month and 36-month overall survival rates were 62.8% and 24.1%, respectively. Survival analysis showed that skull invasion at initial diagnosis (P=0.029) and disease progression (PD) after primary treatment of the primary lesion (P=0.041) were adverse prognostic factors. The treatment response to central nervous system metastatic lesions was a key prognostic indicator: patients achieving complete remission (CR) or partial remission (PR) (27.9%) had significantly longer survival than those with PD (P=0.034), and the survival advantage was mainly observed in the late follow-up period. The risk of recurrence or progression was highest within the first 6 months after central nervous system metastasis.

Conclusion: For pediatric patients with high-risk neuroblastoma and central nervous system metastasis, comprehensive treatment including teniposide-based chemotherapy combined with intrathecal injection can improve the survival prognosis of NB patients with CNS metastasis, and treatment response is a key predictor of long-term survival in these patients.

Abstract 299

A multi-center cases summary for stage L1 neuroblastoma with MYCN amplification

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Purpose: The treatment protocol for stage L1 neuroblastoma with MYCN amplification is still controversial, This study retrospectively summarizes its clinical characteristics and prognosis to provide a reference for future treatment.

Methods: This study included data from multiple children's tumor centers, and retrospectively analyzed stage L1-stage neuroblastoma cases from January 2017 to July 2025, which were accompanied by MYCN amplification. The general information, examination and laboratory data,pathological classification, treatment plan, and follow-up prognosis of the patients were collected.

Results: A total of 14 pediatric patients were enrolled, including 9 males and 5 females. The age range was from 1 day to 99 months, with a median age of 19.7 months. The tumor was located in the posterior mediastinum in 2 cases (14.3%) and in the retroperitoneum or adrenal gland in 12 cases (85.7%), with 6 cases on the right side and 6 cases on the left side. Tumor size ranged from 0.320.330.28 cm to 11.5109 cm.LDH levels were elevated to varying degrees in all patients, with 8 cases (57.1%) <500 U/L and 6 cases (42.8%) >500 U/L. NSE was positive in 13 cases (92.8%). Four patients had combined 1p deletion. Regarding pathological types, 1 case (7.1%) was nodular neuroblastoma, 13 cases (92.9%) were neuroblastoma, among which 10 cases (76.9%) were poorly differentiated, 1 case (7.1%) was differentiated, and 2 cases (14.2%) were undifferentiated.All 14 patients underwent one-stage surgical resection, including 6 cases treated with laparoscopic or thoracoscopic surgery (one of which underwent robotic surgery) and 8 cases treated with open laparotomy for tumor removal. In terms of treatment strategies, 1 case (7.1%) received a low-risk regimen, 10 cases (71.5%) received an intermediate-risk regimen, and 3 cases (21.4%) received a high-risk regimen. One patient underwent autologous stem cell transplantation and dinutuximab immunotherapy.All patients achieved complete remission (CR). The follow-up time ranged from 1 to 90 months, with a median follow-up time of 38 months.

Conclusion: Patients of L1-stage neuroblastoma with MYCN amplification have a favorable clinical prognosis. And if completely resected by surgery, a medium or low risk chemotherapy regimen can be given. According to the condition of the patient, chemotherapy should be carefully carried out in accordance with the high-risk group scheme.

Abstract 300

EFFICACY AND SAFETY OF SIROLIMUS IN THE MANAGEMENT OF PEDIATRIC VASCULAR TUMORS AND MALFORMATIONS: A PROSPECTIVE STUDY

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Purpose: Complex pediatric vascular anomalies cause significant morbidity and often resist conventional therapies. The mTOR pathway contributes to abnormal angiogenesis in these lesions. Sirolimus is a promising targeted therapy; however, data on efficacy, safety, and long-term outcomes in children remain limited. This study evaluates the efficacy, safety, and pharmacokinetics of sirolimus in a pediatric cohort with complex vascular anomalies.

Methods: This observational study evaluated pediatric patients with vascular anomalies treated with oral sirolimus. Baseline clinical and laboratory data were recorded with therapeutic drug monitoring. Disease response was assessed clinically and radiologically at 3 and 6 months, and response was categorised as complete response (CR), partial response (PR), stable disease (SD), or loss to follow-up (LTFU). Adverse events were systematically documented and graded.

Results: Twenty-five pediatric patients (median age 9 years; 56% female) with complex vascular anomalies were evaluated. Venolymphatic malformations were most common (76%), followed by Kaposiform hemangioendothelioma, hemangioma, lymphangioma, PHACE-associated venolymphatic malformation, and fibroadipose vascular anomaly. Lesions were predominantly located in the head and neck (60%). Sclerotherapy was the most frequent prior treatment (44%). Median sirolimus trough level at 2 weeks was 6.88 mcg/L, increasing to 12.1 mcg/L after dose adjustment. Among patients with adverse events, median drug level was 9.44 mcg/L. At 3 months, PR was observed in 60%, stable disease in 24%, with three lost to follow-up and one death. At 6 months, 88% of evaluable patients achieved PR, with varying degrees of size reduction. Sirolimus was generally well tolerated; 44% had no adverse events. The main toxicity was mucositis (mostly grade 1–2), with rare events including hypertriglyceridemia and tuberculosis reactivation.

Conclusion: Sirolimus is an effective, well-tolerated therapy for pediatric vascular tumours and malformations, with a majority having a partial response at 6 months. Therapeutic drug monitoring ensures safety, with mainly mild mucositis, supporting its early use in refractory vascular anomalies

Abstract 301

EWING SARCOMA OF THE RIBS: OUTCOMES BASED ON RIB CHARACTERISTICS.

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Purpose: Ribs comprise the most common component of chest wall Ewing Sarcoma in children. Limited information is present regarding the characteristics of the ribs involved and their association with outcomes. We describe the various components of the rib involved and correlate them with outcomes.

Methods: Clinical characteristics and outcome data of patients with Ewings sarcoma of ribs treated between 2006 to 2023 were retrieved from a prospectively maintained institutional database. Ribs were classified based on their anatomical curvature into anterior, lateral and posterior segments; descending levels into upper (1st-4th), middle (5th-8th) and lower (9th-12th); the number of ribs involved into single or multiple. Neoadjuvant chemotherapy followed by surgery and adjuvant chemotherapy/radiation was offered.

Results: The median age of 117 patients ranged between 1-16 years (median 11 years) with a male (64%) dominance. Right-sided lesions (61%) and single rib (60.7%) were frequently involved. Middle level ribs (49%) and lateral segments (56%) were primarily affected. A partial or complete excision of single rib (59%) or multiple ribs (41%) with meshplasty (50%) was performed. The resection margins were involved in 8 patients. Complete or more than 90% tumor necrosis was seen in 45%. Median follow-up duration was 53 months. The 5-year event-free and overall survival is 54.6% and 56% respectively. 40 patients had relapsed with virtually similar number of local and distant relapses. The prognostic factor associated with poor event-free survival includes involvement of multiple ribs (42.1% vs. 42%, p=0.007) and upper ribs (43.7% vs. 75.5% and 72%, p=0.01). Operative factors for inferior outcomes included resection of multiple ribs, adjacent lung parenchyma and positive surgical margins.

Conclusion: Ewing sarcoma frequently affected right-sided, single, lateral segments of middle level ribs in this study. Multiple and upper ribs involvement were associated with adverse outcomes. Resection of multiple ribs and adjacent lung parenchyma also affected the outcomes.

Abstract 302

Large Solid Pseudopapillary Neoplasm of the Pancreatic Head with Hepatic Metastasis in an Adolescent Female: A Case Report

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Purpose: Solid pseudopapillary neoplasm (SPN) is a rare pancreatic tumor with low-grade malignant potential, accounting for 0.13–2.7% of all exocrine pancreatic neoplasms. Pediatric cases constitute approximately 8–12% of pancreatic tumors. While most SPNs are localized, 10–15% may exhibit local invasion or metastasis to the liver, lung, mesentery, omentum, or peritoneum. Hepatic metastasis is extremely rare in pediatric patients. Accurate histopathologic evaluation, including immunohistochemistry (IHC), is essential for definitive diagnosis.

Methods: We present a case of an 18-year-old female incidentally found to have an abdominal mass. Diagnostic workup included ultrasound and contrast-enhanced abdominal CT to evaluate tumor size, location, and potential metastasis. The patient underwent complete surgical excision of both the pancreatic and hepatic lesions. Pathologic and IHC analyses, including Beta-catenin staining, were performed to confirm tumor type and assess metastatic involvement.

Results: CT imaging revealed a heterogeneous mass measuring 10.4 × 9.7 × 8.0 cm in the head of the pancreas, with irregular and partially well-defined margins. A hypodense lesion with poorly defined borders was noted in the SIII segment of the liver, suggestive of metastasis. The patient underwent complete surgical excision of both lesions. Histopathology demonstrated pseudopapillary structures with monomorphic tumor cells surrounding fibrovascular cores. IHC staining showed strong nuclear and cytoplasmic Beta-catenin positivity in both the pancreatic tumor and hepatic lesion, confirming SPN with hepatic metastasis. Resection margins were negative, and no perineural invasion was observed.

Conclusion: This case highlights that SPN, although generally a low-grade tumor with favorable prognosis, can rarely metastasis to the liver in young patients. Accurate histopathologic evaluation, particularly Beta-catenin IHC, is critical for diagnosis. Early recognition, comprehensive evaluation, and complete surgical resection of both primary and metastatic lesions are essential to achieve optimal long-term outcomes. Careful postoperative follow-up is recommended due to the rare possibility of recurrence.

Abstract 303

Massive pericardial and pleural effusion as the initial manifestation of mediastinal germ cell tumor

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Purpose: Massive pericardial and pleural effusions are rare initial manifestations of germ cell tumors, particularly in pediatric. Establishing an accurate diagnosis of solid tumors in resource-limited settings remains difficult and often requires a prolonged period. These delays result from several factors, including patients arriving late to healthcare facilities and delayed referral due to low suspicion for malignancy. Here we report a case of mediastinal germ cell tumor with concomitant pericardial and pleural effusion.

Methods: A referral case of a 12-year-old boy with right pleural effusion and suspected thoracic mass, presenting with facial edema, dyspnea, orthopnea, and chest discomfort. A thorax CT scan found a cystic mass on the right mediastinum compressing the lung and superior vena cava. Bedside ultrasound confirmed pleural effusion and a ‘swimming heart’ sign, indicating massive pericardial effusion with right atrial and ventricular collapse. Superior vena cava syndrome and impending cardiac tamponade was present. Pleural and pericardial drainage yielded 500 mL and 270 mL of serohemorrhagic fluid, respectively. Following the procedure, respiratory effort worsened, requiring mechanical ventilation. During PICU admission, the patient had persistent facial edema and required high ventilatory pressure to maintain an adequate pCO_2 level. Hypotension occurred and was managed with norepinephrine and epinephrine. Laboratory results showed elevated beta HCG (22.9 mIU/mL) and alpha fetoprotein (46,220 IU/mL). Multidisciplinary team discussion was conducted and tumor debulking was considered as the definitive procedure, but the patient was unstable. The hemato-oncological team proposed germ cell chemotherapy protocol (etoposide, cisplatin, ifosfamide) to reduce tumor size. The chemotherapy was commenced at a reduced dose and later escalated to a full dose, despite pending fluid cytopathology results. His condition continued to deteriorate. He developed supraventricular tachycardia. The resuscitation was once successful, but later his parents provided do-not-resuscitate consent. The patient died after 6 days in PICU. Pleural fluid cytopathology revealed suggestive findings of germ cell tumor.

Results: Malignancy is an uncommon etiology of massive pericardial and pleural effusions. Early detection and intervention may be lifesaving, but clinical management can be challenging. Mechanical ventilation is often necessary to maintain oxygenation; however, positive pressure ventilation may aggravate mediastinal mass syndrome by precipitating obstructive shock. In our setting, advanced modalities such as extracorporeal membrane oxygenation were not available, limiting further therapeutic options.

Conclusion: This case illustrates that oncologic emergency requiring rapid multidisciplinary management. Although rare, malignant causes should be considered in patients presenting with massive pericardial and pleural effusions to enable early diagnosis and timely intervention.

Abstract 304

Bioinformatics Analysis of Neuroactive Substance-Related Genes in Neuroblastoma and Study on the Biological Function and Targeted Drugs of Key Gene Shc3

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Purpose: Neuroblastoma (NB) is a common childhood extracranial solid tumor, accounting for 8%-10% of childhood malignancies and a major cause of cancer-related death. High-risk NB has a poor prognosis (5-year survival <50%) despite multimodal therapy. Neuroactive substances and their key gene Shc3 are linked to neurogenic tumor progression, but their regulatory mechanisms and targeted strategies in NB are unclear. This study explored neuroactive substance-related genes, clarified Shc3’s function in NB, and screened targeted drugs to provide new diagnostic and therapeutic ideas.

Methods: Neuroactive substance-related genes were obtained from MSigDB, and differentially expressed ones (DE-NRGs) in NB were screened via R limma

package. A prognostic model was constructed with 10 machine learning algorithms (101 combinations) and validated. Shc3 was identified as a key gene, and its expression was verified by immunohistochemistry, qPCR and Western blot. Stable Shc3 cell models were established, and its effects on NB cell functions were detected. Shc3's 3D structure was from AlphaFold; targeted compounds were screened via molecular docking, with stability and anti-tumor activity verified, and combined drug therapy synergy was explored.

Results: Eighty-eight DE-NRGs were screened, and the constructed Cox_RSF prognostic model showed good predictive efficacy. Shc3 was highly expressed in high-risk NB, correlated with poor prognosis, and promoted NB cell proliferation and migration. Targeted compounds (e.g., ENMD-2076) bound stably to Shc3 and exhibited in vitro anti-tumor activity; combined drug therapy further enhanced this anti-tumor efficacy.

Conclusion: Shc3 is a key gene for poor prognosis of NB, which can be used as a potential target for NB targeted therapy, and the screened targeted drugs and their combined therapy provide a new strategy for NB treatment.

Abstract 305

Pediatric Neuroblastoma: Clinical Feature Analysis, Treatment Dilemma Breakthrough and Optimization Strategy Exploration

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Purpose: Pediatric neuroblastoma, a prevalent extracranial solid neoplasm in children (accounting for 6%-10% of childhood malignancies), poses a substantial menace to the lives of the young. This research endeavors to augment the treatment efficacy and survival rates through an exhaustive dissection of its clinical, pathological, and therapeutic dimensions.

Methods: Utilizing the retrospective case study approach, data spanning from 2022 to August 2024, inclusive of clinical records, laboratory test findings, imaging data, and treatment regimens, were amassed from Hebei Children's Hospital. Subsequent to a meticulous curation of missing values and outliers, and the implementation of standardization procedures, both descriptive statistics and advanced analytical techniques were employed to probe the correlations between symptoms and the disease entity.

Results: Clinically, manifestations are variegated, with abdominal masses being conspicuously prevalent. Pathologically, a multiplicity of types and grades is discernible. The multimodal treatment strategies are beleaguered by tumor heterogeneity, adverse side effects, drug resistance, and recurrence predicaments.

Conclusion: A comprehensive clinical panorama is presented. Recommendations entail bolstering multidisciplinary collaboration, instituting a full-course management paradigm, and assimilating novel technological advancements. Future research efforts should be concentrated on unearthing the pathogenic and drug resistance mechanisms, broadening clinical trials, and harnessing intelligent data to refine prognostic estimations, with the aspiration of revolutionizing the diagnostic and treatment modalities for pediatric neuroblastoma.

Abstract 306

Feasibility of Left Subcostal Single-Incision Laparoscopic Surgery for Pediatric Left Retroperitoneal Space-Occupying Lesions: A Preliminary Report of 12 Cases from a Single Center

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Purpose: Minimally invasive surgery has become integral to pediatric surgical oncology. However, conventional multiport laparoscopy and transumbilical single-incision approaches encounter limitations when accessing some left retroperitoneal regions due to suboptimal visualization, instrument triangulation challenges, and physical interference in lateral positioning. To evaluate the technical feasibility, safety, and preliminary efficacy of left subcostal single-incision laparoscopic surgery (LS-SILS) in the right lateral position for various left retroperitoneal space-occupying lesions in children.

Methods: This single-center, retrospective, observational case series analyzed 12 consecutive pediatric patients with left retroperitoneal space-occupying lesions who underwent LS-SILS between January and December 2025. All procedures were performed through a 1.5–2.0 cm single incision in the left subcostal region with the patient positioned at approximately 70° right lateral decubitus. Perioperative outcomes and pathological results were analyzed.

Results: All 12 procedures were successfully completed via the single incision without conversion to open surgery or additional trocar placement. Pathological diagnoses included: extralobar pulmonary sequestration (n=6), solid pseudopapillary neoplasm of the pancreas (n=2), adrenal neuroblastoma (n=1), retroperitoneal mature teratoma (n=1), lymphatic malformation with infection (n=1), and adrenal ganglioneuroma with retroperitoneal metastasis (n=1). Median operative time was 83.5 minutes, and median estimated blood loss was 7.5 mL. 9 patients resumed oral intake on the day of surgery, with a median postoperative hospital stay of 3 days. No pancreatic fistula occurred in the 2 pancreatic surgery patients. Notably, one patient with adrenal ganglioneuroma and retroperitoneal metastasis underwent complete single-stage resection of both lesions through the same incision, with uneventful recovery.

Conclusion: LS-SILS in the right lateral position is a safe and feasible innovative minimally invasive technique for pediatric left retroperitoneal lesions. Through optimized positioning and incision design, it provides clear exposure and precise access to both left retroperitoneal and retroperitoneal lesions, demonstrating excellent technical expandability and significant clinical potential.

Abstract 307

Childhood Soft Tissue Sarcoma at Yangon Children's Hospital: A Resource-Limited Single-Center Experience (2013–2025)

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Purpose: Childhood soft tissue sarcomas are a heterogeneous group of malignant tumors arising from mesenchymal tissues and represent an important proportion of pediatric solid cancers. In low- and middle-income countries, outcomes are often affected by delayed diagnosis, advanced disease at presentation, and limited access to multimodal treatment. This study aimed to describe the clinical spectrum and treatment outcomes of childhood soft tissue sarcomas treated at Yangon Children's Hospital over a 13-year period.

Methods: A retrospective descriptive study was conducted including children diagnosed with soft tissue sarcoma between January 2013 and December 2025 at Yangon Children's Hospital. Treatment approaches included combinations of chemotherapy, surgery, and radiotherapy.

Results: A total of 257 patients were identified, including 131 males and 126 females. The cohort comprised 117 cases of Rhabdomyosarcoma (RMS), 80 non-rhabdomyosarcoma soft tissue sarcomas, and 60 cases of Ewing Sarcoma. Metastasis at diagnosis was observed in 190 patients (73.9%), indicating late presentation in the majority of cases. Among RMS patients, embryonal RMS accounted for 77 cases (65.8%), followed by alveolar RMS in 28 cases (23.9%). In the RMS group, 51 patients (43.6%) defaulted from treatment, 15 survived, 50 died, and one remained on therapy. In the non-RMS group, common histological types included fibrosarcoma (14 cases, 17.5%) and desmoid-type fibromatosis (8 cases, 10%). Outcomes in this group included 46.2% treatment default, 19 survivors, 17 deaths, and 7 patients still receiving treatment. Among Ewing sarcoma patients, 10 survived, 32 died, 16 defaulted, and 2 remained on treatment.

Conclusion: Childhood soft tissue sarcomas at Yangon Children's Hospital present a substantial clinical challenge, with high rates of metastatic disease and treatment abandonment. Strengthening early diagnosis, improving access to multidisciplinary care, and implementing strategies to reduce treatment default are essential to improve survival outcomes in resource-limited settings.

Abstract 308

Osteosarcoma in Children and Adolescents at Yangon Children's Hospital: A Resource-Limited Single-Center Experience (2013–2025)

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Purpose: Osteosarcoma is the most common primary malignant bone tumor in children and adolescents and accounts for approximately 2–5% of pediatric

malignancies worldwide. Survival has significantly improved in high-income countries through multimodal therapy including chemotherapy and limb-salvage surgery. However, outcomes in low- and middle-income countries remain poorer due to delayed diagnosis, limited access to specialized care, and treatment abandonment. This study aimed to describe the clinical characteristics, management, and outcomes of pediatric osteosarcoma treated at Yangon Children's Hospital over a 13-year period.

Methods: A retrospective descriptive study was conducted at Yangon Children's Hospital including all children and adolescents with Osteosarcoma between January 2013 and December 2025

Results: A total of 59 patients were identified during the study period, including 27 males and 32 females. Most patients presented with painful bony swelling. The femur was the most frequently affected primary site. Metastasis at diagnosis was present in 30 patients (50.8%), with the lung being the most common metastatic site. All patients underwent surgical management with limb amputation and received chemotherapy according to the EURAMOS-1 Protocol. Metastasectomy procedures were not performed. Histopathological examination showed that conventional osteosarcoma was the most common subtype. Regarding clinical outcomes, 14 patients (23.7%) survived, 24 patients (40.7%) died, 17 patients (28.9%) defaulted from treatment, and 4 patients remained on therapy at the time of analysis. Local recurrence at the primary site occurred in two patients, while three patients developed relapse at secondary sites including the lung and humerus.

Conclusion: This single-center experience highlights the major challenges in managing pediatric osteosarcoma in a resource-limited setting, including high rates of metastatic disease at presentation and treatment abandonment. Strengthening early diagnosis, improving access to multidisciplinary cancer care, and addressing barriers to treatment adherence are essential to improve survival outcomes

Abstract 309

Malignant Epithelioid Hemangioendothelioma of bone in a Child: A Rare Case Report

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Purpose: Background: Epithelioid Hemangioendothelioma (EHE) is a rare vascular tumor with intermediate malignant potential that arises from endothelial cells. It occurs predominantly in adults and commonly involves the liver, lung, and soft tissues. Primary involvement of bone in children is extremely uncommon, and diagnosis can be challenging due to its nonspecific clinical and radiological features.

Methods: Case Presentation: We report a 5-year-old boy who presented with pain in the right hip joint and swelling. Radiographic evaluation with hip X-ray revealed a pathological fracture involving the neck of the right femur. Initial laboratory investigations showed elevated alkaline phosphatase (ALP) of 543 U/L, while other laboratory parameters were within normal limits.

Results: Further evaluation with tissue biopsy demonstrated features consistent with malignant epithelioid hemangioendothelioma. Immunohistochemical analysis showed strong positivity for vimentin (100%) and CD34 expression in normal blood vessels, immature vessels, and neoplastic cells, supporting endothelial differentiation. Pan-cytokeratin was negative, while S100 showed scattered positivity in approximately 8% of tumor cells. These findings confirmed the diagnosis of malignant epithelioid hemangioendothelioma of the proximal femur. The patient was initiated on systemic chemotherapy. The first cycle of the VDC protocol (vincristine, doxorubicin, and cyclophosphamide) was administered while awaiting planned radiotherapy for local disease control.

Conclusion: The clinical and radiological presentation initially raised concern for more common malignant bone tumors; however, histopathological examination revealed malignant epithelioid hemangioendothelioma. Malignant epithelioid hemangioendothelioma of bone is a rare entity in the pediatric population. This case highlights the importance of considering vascular tumors in the differential diagnosis of pathological fractures in children and emphasizes the role of histopathology and immunohistochemistry in establishing the diagnosis.

Abstract 310

Secondary tumors in children of Moscow region

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Purpose: Second malignant neoplasms (SMNs) represent one of the most severe late adverse effects of anticancer therapy in childhood. Aim to evaluate the structure of second malignant neoplasms in childhood cancer patients.

Methods: A retrospective analysis of children and adolescents diagnosed with SMNs in the Moscow region between 2012 and 2025 identified 9 cases of malignant neoplasms.

Results: Hematological malignancies predominated among primary cancers, accounting for 5 of 9 cases. Solid extracranial tumors were observed in 3 cases, and central nervous system (CNS) tumors in 1 case. Primary hematological malignancies included acute lymphoblastic leukemia (ALL) in 4 patients and Burkitt lymphoma in 1 patient. Primary solid tumors were represented by neuroblastoma (n=2), embryonal rhabdomyosarcoma (n=1), and one CNS tumor (ependimoma). All patients received polychemotherapy for primary malignancy; 5 of 9 patients underwent radiotherapy with total doses ranging from 12 to 54 Gy, and one patient received autologous hematopoietic stem cell transplantation. The median age at primary cancer diagnosis was 49 months (range 13–83). Among SMNs, hematological malignancies were diagnosed in 6 cases, including acute myeloid leukemia (n=5) and acute lymphoblastic leukemia (n=1). One patient developed osteosarcoma, and two patients were diagnosed with CNS tumors (glioma). The median age at SMN diagnosis was 130 months (range 46–182). The median latency period between primary cancer and SMN development was 79 months (range 33–145). At the time of analysis, 5 of 9 patients were alive, with a median follow-up of 17 months. Four patients died: two due to SMN progression and two from septic complications during chemotherapy. One patient with ALL was diagnosed with Li–Fraumeni syndrome.

Conclusion: This study highlights that patients receiving treatment in LMIC at centralized institutes such as TMH achieve favorable survival outcomes, with results aligning closely to international standards. Centralized registration and protocol driven management ensured consistency in care delivery and improved survival across cohorts. These findings reinforce the importance of specialized centers in optimizing outcomes for pediatric hepatic tumors in resource constrained settings.

Abstract 311

CONGENITAL MESOBLASTIC NEPHROMA: THE RESULTS OF THE RETROSPECTIVE ANALYSIS

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Purpose: Congenital mesoblastic nephroma (CMN) is a rare pediatric renal tumor with low malignant potential that occurs in 3% of cases of kidney tumors in children and has a favorable clinical outcome.

Methods: A retrospective analysis of 32 patients aged 0–18 years with histologically confirmed CMN treated at the national center was performed from 1.2012 to 01.2024. Demographic, clinical, histological, and treatment data were evaluated. Therapy was provided according to SIOP-RTSG protocols. Statistical analysis was completed on 01.01.2025.

Results: We recruited 32 patients with CMN. The median age at diagnosis was 1.6 months (range: birth–5.6 months). Male:female ratio was 1:1. CMN was detected during routine fetal ultrasound in 11/32 (35%) cases.The distribution according to SIOP local stages was as follows: I – 6/32(19%), II – 20/32(62%), III – 6/32(19%). Histological types were classic -16(50%), cellular - 12(37.5%), and mixed - 4(12.5%). Median age at diagnosis did not differ significantly between CMN types (overall p = 0.059). The median tumor volume was 64 cm3 (range 3.9-749 cm3). The largest tumor volume was observed in the mixed type (226 cm^3) compared with the cellular (168 cm^3) and classic types (24cm^3) (overall p = 0.002).. Rearrangement in the ETV6 gene was detected in 12/15 (80%) cases: 10/12 (83%) cases with the cellular type and 2/3 (67%) cases with the mixed type.

Four of 32 patients (12.5%) received 4 weeks of neoadjuvant AV chemotherapy. Surgical treatment included 31 nephrectomies (96.9%) and 1 kidney-sparing resection (3.1%) in a patient with HNF1B-associated nephropathy. EFS was 87.5 ± 6.0% (four local relapses). OS was 93.8 ± 4.3% (two patients died of treatment-related complications.

Conclusion: CMN in our cohort predominantly affected infants in the first 3 months of life, was most often classic or cellular. Overall, outcomes were favorable, with high EFS and OS despite local relapses and limited treatment-related mortality.

Abstract 312

KMT5A suppresses ferroptosis in rhabdomyosarcoma through H4K20me1/GTSE1 axis-mediated p53 ubiquitination and degradation

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Purpose: KMT5A is a lysine methyltransferase that specifically catalyzes histone H4K20 monomethylation (H4K20me1) and is overexpressed in various cancers. However, its role in rhabdomyosarcoma (RMS) remains unknown. This study aimed to investigate the mechanism by which KMT5A regulates ferroptosis in RMS.

Methods: RMS cell lines (RD and RH30) were used. Unbiased screening of 25 epigenetic inhibitors was performed. KMT5A expression was analyzed in public databases and clinical samples. Stable KMT5A-knockdown cells were established using shRNA. The levels of lipid peroxidation (C11-BODIPY), Fe^{2} (FerroOrange), and GSH were detected using respective assays. Transcriptomic analysis was performed after KMT5A knockdown. CUT&Tag and ChIP-qPCR were used to detect H4K20me1 enrichment at GTSE1 promoter. Co-immunoprecipitation and ubiquitination assays were used to examine protein interactions and p53 degradation. Western blot and RT-qPCR were used to detect gene expression levels. In vivo, subcutaneously implanted tumors were established using nude mice.

Results: KMT5A was significantly overexpressed in RMS and correlated with poor prognosis. KMT5A knockdown or UNC0379 treatment induced cell death in RMS cells, accompanied by increased lipid peroxidation and Fe^{2} levels and decreased GSH levels. Ferroptosis-related protein expression was also altered. KMT5A knockdown–induced cell death could be reversed by the ferroptosis inhibitor Ferrostatin-1. Transcriptomic analysis showed enrichment of the p53 signaling pathway, and GTSE1 was identified as a key downstream target. KMT5A knockdown reduced H4K20me1 enrichment at the GTSE1 promoter and decreased GTSE1 expression at both mRNA and protein levels. GTSE1 overexpression rescued KMT5A knockdown–induced ferroptosis. Furthermore, GTSE1 recruited MDM2 to promote p53 K48-linked polyubiquitination and degradation, thereby relieving p53-mediated transcriptional repression of SLC7A11. In vivo, KMT5A inhibition suppressed tumor growth through ferroptosis induction.

Conclusion: KMT5A suppresses RMS ferroptosis via the H4K20me1/GTSE1/p53 axis, representing a potential therapeutic target.

Abstract 313

Sequential intravenous and intracerebroventricular GD2-NKG2D multi-targeting CAR-T therapy induces durable remission in relapsed neuroblastoma with brain metastases: a case report

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Purpose: High-risk neuroblastoma (NB) with central nervous system (CNS) involvement and MYCN and ALK co-amplification portends an exceptionally poor prognosis, with median survival under 2 years. GD2-targeted CAR-T therapy has shown preliminary feasibility, but tumor heterogeneity limits complete tumor eradication. We developed a novel, third-generation, multi-targeting CAR-T product, DeepTag-GD2-NKG2D Bicephali, targeting both GD2 and NKG2D ligands, and evaluated sequential intravenous and intracerebroventricular (ICV) delivery.

Methods: A 1-year-old boy with high-risk stage IV NB (MYCN and ALK co-amplification) developed CNS relapse after multimodal therapy. Following surgery, salvage chemo-radiotherapy, and lorlatinib (best response: partial remission), he enrolled in a phase 1 study assessing the safety and efficacy of GD2-NKG2D multi-targeting CAR-T therapy for relapsed or refractory NB (NCT06836505). After lymphodepleting chemotherapy, he received intravenous GD2-NKG2D multi-targeting CAR-T cells (1×10^{6}/kg), followed by repeated ICV infusions via Ommaya reservoir every 4 weeks without lymphodepletion (11 doses; 1×10^{7}-5×10^{7} cells per dose). Response was assessed by brain MRI, CAR-T cell quantification in blood and cerebrospinal fluid (CSF), and cytokine profiling.

Results: Complete remission (CR) was achieved after three ICV infusions and maintained through data cutoff (event-free survival 22 months post-relapse). Grade 3 cytokine release syndrome (CRS) occurred after intravenous infusion, resolving with tocilizumab and ruxolitinib-based intervention. Following ICV dosing, only grade 1 CRS was observed after six infusions (54.5%) and no immune effector cell-associated neurotoxicity syndrome was observed. CAR-T cells demonstrated robust expansion in peripheral blood and CSF, with sustained CSF persistence (>30 days). ICV administration induced the elevation of cytokines (IFN-γ, IL-6, and IL-10) in CSF, supporting local CAR-T activation and cytotoxic activity.

Conclusion: Sequential intravenous and ICV GD2-NKG2D multi-targeting CAR-T therapy achieved durable CNS remission with manageable toxicity, supporting a muti-targeting, locoregional delivery strategy for high-risk NB with CNS metastases. These early signals merit validation in larger prospective cohorts to clarify long-term efficacy and safety.

Abstract 314

Osteosarcoma and Ewing's sarcoma of the head & neck in pediatric practice, surgical treatment. Experience of Dmitry Rogachev National Medical Research Center Of Pediatric Hematology, Oncology and Immunology

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Purpose: Osteosarcoma and Ewing's sarcoma are malignant tumors that develop from bone tissue and are rare tumors in pediatric practice. According to the various data, the primary localization of these tumors in head & neck region occurs from 1% to 9.8%, however, they most often occur in the upper and lower extremities and pelvic bones.

Methods: A retrospective analysis of surgical interventions and long-term results for osteosarcoma and Ewing's sarcoma of maxillofacial bone localization was carried out, including both an analysis of surgical local control and reconstructive approaches of this group of patients. From 2015 to January 2026, 33 patients with primary osteosarcoma and Ewing's sarcoma with localization of the tumor of the maxillofacial region received surgical treatment at Dmitry Rogachev NMRC of PHOI. All patients with osteosarcoma were treated according to the EURAMOS protocol, with Ewing's sarcoma - according to the Euro-Ewing 2008/2012 protocols. Also, during this period, three patients were treated for secondary osteosarcoma after radiotherapy.

Results: The lesion of the mandible occurred in 13 patients (in 6 clinical cases - osteosarcoma, in 7 clinical cases - Ewing's sarcoma). Upper jaw lesion in 10 patients (osteosarcoma in 4 clinical cases, Ewing's sarcoma in 6 patients). Skullbase localization of the tumor occurred in 6 patients (1 patient with osteosarcoma, 5 patients with Ewing's sarcoma). Temporal bone lesion was found in 2 clinical cases (histologically, Ewing's sarcoma was present in all patients). In 2 patients, the tumor affected the frontal bone (2 patients with Ewing's sarcoma). The report presents the results of the effect of radiation therapy on the reconstructive treatment of patients with osteosarcoma and Ewing's sarcoma of head and neck region.

Conclusion: Modern technologies, such as individual planning using resection STL-templates, microsurgical technologies, the use of endoprosthesis technologies with a sliding “growing” mechanism followed by complex maxillofacial

rehabilitation, allow to achieve functional and aesthetic results of surgical treatment with good clinical outcomes.

Abstract 315

Combination of anti-GD2 with anti-PD-1 enhances tumor responses and elicits T cell memory via inducing immunogenic cell death

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Purpose: A chimeric anti-GD2 antibody has been shown to significantly improve the survival of high-risk neuroblastoma. However, ~40% of patients still relapse and succumb to disease, necessitating new strategies to improve the efficacy of anti-GD2. We aim to boost its efficacy by combining with anti-PD1.

Methods: The efficacy of combining anti-GD2 with anti-PD-1 was assessed in A/J and C57BL/6 mice bearing GD2 (+) neuroblastoma NXS2 and lymphoma EL4, respectively. Tumor volume and mouse survival were monitored. Immune cell profiles and immunogenic cell death markers were determined by flow cytometry.

Results: Anti-PD-1 boosted efficacy of anti-GD2 in NXS2- and EL4-bearing mice, with increased NKG2D+ NK cells in spleen/TME and decreased MDSC in spleen. Long term surviving mice were protected from tumor rechallenge, which was mediated by memory T cells, in response to immunogenic cell death of tumor cells.

Conclusion: The combination of anti-GD2 with immune checkpoint blockade showed enhanced anti-tumor responses. Such combination can induce immunogenic cell death of tumor cells to generate tumor-specific immunological memory CD4 and CD8 T cells, leading to augmented and durable anti-cancer efficacy.

Abstract 316

Laparoscopic Surgery for Pediatric Kidney Tumors

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Purpose: MIS is expanding in pediatric oncology, yet laparoscopic nephrectomies and nephron-sparing surgeries remain rare for kidney masses. Analyzing institutional experience and expanding indications is crucial. To describe technical features of laparoscopic nephrectomies and resections for kidney tumors.

Methods: From January 2018 to February 2026, 66 patients underwent laparoscopic surgery for kidney masses (34 females, 32 males). Median age varied by procedure: nephrectomy 49 months, resection 81.7 months. All patients underwent contrast-enhanced MRI/MSCT. Treatment followed SIOP 2016-RTSG protocol.

Results: Nephrectomy was performed in 41 (62%) patients, resection in 25 (38%). Median blood loss: 10 ml vs. 50 ml. ICU stay median: 1 day for both. Drain removal: day 1 (nephrectomy) vs. day 4 (resection). Event-free survival: nephrectomy 78.49±4.01 months (n=3); resection group (85.5±5.66 months).

Conclusion: MIS for pediatric kidney tumors offers significant advantages: early rehabilitation, reduced blood loss, shorter hospitalization, and cosmetic benefits. These procedures adhere to core oncological surgery principles.

Abstract 317

A Rare Gaze: Congenital Ewing Sarcoma in the Neonate

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Purpose: Ewing sarcoma comprises a range of aggressively malignant neuroectodermal tumors. Congenital Ewing sarcoma affecting newborns (≤ 28 days of age) is uncommon. Currently, there is no study published regarding the occurrence of primary orbital ES in the neonatal age group.

Methods: This is a case report of a 13-day-old female neonate who was admitted to a pediatric tertiary institution due to proptosis of the left eye. Biopsy revealed a malignant small round cell neoplasm.

Results: IHC was positive for CD99, NKX2.2, and Synaptophysin, confirming the diagnosis of ES. The treatment plan proposed after surgery was a combination of 17 cycles of dose-reduced chemotherapy with the VACD-IE regimen with focal radiotherapy.

Conclusion: A multidisciplinary approach in the treatment includes: surgical, radiotherapy, and adjuvant and neoadjuvant chemotherapy. The survival rate remains low in the neonates, and currently, there is no standard treatment modality established in the neonatal age group.

Abstract 318

Update on Tumors Associated with DICER1 Gene Alterations

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Purpose: DICER1 gene associated tumors represent a cluster of neoplasms, and DICER1 syndrome was a genetic tumor syndrome. The study systematically reviews research on these tumors, with a focus on their pathogenic mechanisms, clinical- pathology features, and the standardized surveillance strategies

Methods: The DICER1 gene related tumors most follow a modified two-hit hypothesis. We elaborated on the features of pleuropulmonary blastoma, thyroid follicular nodular disease, ovarian Sertoli-Leydig cell tumor, cervical embryonal rhabdomyosarcoma, pediatric cystic nephroma, etc.

Results: PPB has three types; TFND, DICER1 syndrome’s most common phenotype; SLCT features early onset; cERMS has a relatively indolent course; pCN is correlated with germline mutations. Stratified surveillance protocols for patients and their first/second-relatives cover related system as recommendations.

Conclusion: This review enhances us understanding of DICER1 gene related tumors. It offers a reference for the early screening, accurate diagnosis and standardized surveillance of DICER1 syndrome, thus alleviating the individual and social health burdens induced by such neoplasms.

Abstract 319

The Ultimate Easter egg: A Hidden Mixed-Germ Cell Tumor in an 8-Year-Old’s Abdomen

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Purpose: Malignant ovarian germ cell tumors are rare in children and often present with vague abdominal pain. We report an 8-year-old with Stage IC mixed germ cell tumor to highlight the importance of early tumor marker testing and imaging in persistent pediatric abdominal pain.

Methods: Clinical, laboratory, imaging, operative, and histopathologic data were reviewed. The patient underwent exploratory laparotomy with right salpingo-oophorectomy and staging. Postoperatively, platinum-based chemotherapy (PEB) was administered with serial AFP monitoring.

Results: An 8-year-old presented with 4 months of abdominal pain and a palpable mass. AFP was markedly elevated. Imaging suggested germ cell tumor. Surgery confirmed Stage IC immature teratoma with yolk sac component. AFP declined after PEB chemotherapy.

Conclusion: Persistent abdominal pain in prepubertal girls warrants evaluation for ovarian malignancy. Early AFP testing, imaging, surgical staging, and platinum-based chemotherapy enable timely diagnosis and favorable outcomes in mixed malignant germ cell tumors.

Abstract 320

Dinutuximab Beta in the Treatment of Relapsed and Refractory Sarcomas in Children

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Purpose: Dinutuximab (anti-GD2) targets GD2-expressing pediatric sarcomas (Ewing's 40-90%, higher in relapse). Study of 37 patients showed 75.7% ORR, 83.8% DCR with acceptable toxicity, supporting its use in refractory/relapsed disease.

Methods: The study included 37 patients: osteosarcoma (46%), Ewing's sarcoma (16.2%), rhabdomyosarcoma (18.9%). Refractory disease in 11 patients, relapse in 26. Stage IV in 54.1%, two or more relapses in 57.6%. All received second-line chemotherapy with dinutuximab beta 100 mg/m².

Results: ORR 75.7%, DCR 83.8%. Relapse group: CR 92%, median PFS 16.5 mo, OS 91.7% (1-yr). Refractory group: CR 27%, median PFS 5.7 mo, OS 60% (1-yr). Dinutuximab beta effective in relapsed/refractory pediatric sarcomas.

Conclusion: Dinutuximab beta in children with relapsed and refractory sarcomas improves survival by 20-30% with favorable tolerability. Immunotherapy may be indicated in first-line for incurable disease. Predictive biomarkers of efficacy require further investigation.

Abstract 321

Circulating Tumor Cells in Ewing's Sarcoma and Soft Tissue Sarcomas: Diagnostic and Prognostic Value

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Purpose: Ewing's sarcoma (ES) is an aggressive bone (rarely soft tissue) tumor affecting children and adolescents. Despite most cases being localized, subclinical metastases are nearly universal. Approximately 25% of localized patients relapse. In relapsed/refractory disease, survival is below 30%.

Methods: CTCs detach from tumors, enter blood, and drive metastasis. In 171 patients, CTC levels correlated with treatment response (sensitivity 70.6%, specificity 80.0%). Flow cytometry detects CD99+/CD45- cells, enabling real-time tumor burden assessment and early relapse detection

Results: Flow cytometry offers advantages: applicability to large patient cohorts, real-time CTC quantification, and accessibility. ES cells highly express CD99 while lacking CD45 and CD34, with characteristic vimentin and IGF-1R expression, enabling CTC identification in blood

Conclusion: Our study demonstrates clinical utility. CTC levels clearly correlated with treatment response, showing decline during therapy. Method sensitivity was 70.6%, specificity 80.0%. CTC detection may assess tumor burden, monitor treatment efficacy, reduce relapse risk, and enable early recurrence detection during follow-up

Abstract 322

Intensified Two-Weekly VDI in Pediatric Ewing's Sarcoma: Comparison with Euro-Ewing 2012

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Purpose: Shortening chemotherapy intervals improves outcomes in Ewing's sarcoma (ES). Euro-Ewing 2012 (EE2012) showed superiority of 2-week VDC/IE over 3-week VIDE. Evaluate efficacy of 2-week vincristine/doxorubicin/ifosfamide (VDI) in pediatric ES and compare with EE2012.

Methods: Analysis of 171 ES patients (stages 2A-4B) treated 2008-2022. Median age 12 years. All received induction VDI with 14-day intervals. Stage IV patients more often received high-dose chemotherapy (HDCT)

Results: Overall response 97.7%. Three-year OS 86.2%, EFS 69.7%. Prognosis stage-dependent: 5-year OS 83.0% (stage II) vs 51.4% (stage IV, p=0.002). HDCT improved OS (p=0.021). Our outcomes surpassed EE2012 VIDE (74% OS, 61% EFS) and matched VDC/IE (82% OS, 67% EFS)

Conclusion: Two-week VDI yields high survival in pediatric ES, comparable to intensive international standards. Shortening inter-cycle intervals is critical for successful ES treatment

Abstract 323

Two-Weekly VDI Regimen in Pediatric Ewing's Sarcoma: Results from a Single-Center Study and Comparison with Euro-Ewing 2012

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Purpose: Shortening chemotherapy intervals improves outcomes in Ewing's sarcoma (ES). Euro-Ewing 2012 (EE2012) showed superiority of 2-week VDC/IE over 3-week VIDE. Evaluate efficacy of 2-week vincristine/doxorubicin/ifosfamide (VDI) in pediatric ES and compare with EE2012

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Conclusion: The two-week VDI regimen yields high survival rates in pediatric ES, aligning with modern intensive therapy standards. These findings confirm that treatment intensification through shortened inter-cycle intervals is critical for successful ES outcomes

Abstract 324

Dr

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Purpose: Metastatic rhabdomyosarcoma (mRMS) has poor outcomes despite therapy. While Oberlin score and FOXO1 status are established prognostic markers, their combined utility in refining pediatric risk-stratification remains under-explored.

Methods: Retrospective study of patients aged 0 to 18 with mRMS at Birmingham Children’s Hospital over 10 years. EFS and OS were estimated by Kaplan-Meier and log-rank tests. Univariate Cox regression evaluated prognostic factors.

Results: 22 patients; 50 percent 3-year EFS. FOXO1 fusion (40.9 percent) predicted worse EFS (11 percent vs 77 percent, p less than 0.001). Oberlin 2 to 4 also inferior (p equal to 0.009). 3-tier model EFS: 100, 57, 11 percent (p equal to 0.002). Hazard was 5.5-fold higher per risk step (p equal to 0.004).

Conclusion: FOXO1 and Oberlin status predict survival. Integrating them into a three tier model provides clearer risk separation, facilitating risk adapted therapy for pediatric mRMS. Multicentre validation is warranted.

Abstract 325

RESULTS AND PROGNOSIS IN THE TREATMENT OF OVARIAN DYSGERMINOMA IN CHILDREN AND ADOLESCENTS

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Purpose: To study the effectiveness of immunohistochemical studies in diagnosis and tactics treatment of ovarian dysgerminoma of childhood adolescence.

Methods: We studied 30 patients with ovarian dysgerminoma. Diagnosis was based on standard tumor markers AFP, CA-125, and HCG. Twelve patients were aged 1–14 years and 18 were 15–18 years. After surgery, all underwent immunohistochemical analysis of bcl-2 and the p53 gene.

Results: Among 30 patients, bcl-2 was high in 6 (20%), moderate in 9 (30%), and low in 15 (50%); p53 was high in 8 (26.6%), moderate in 12 (40%), and low in 10 (33.3%). High expression predicted aggressive disease and intensive chemotherapy, while low levels allowed milder treatment.

Conclusion: The performed immunohistochemical studies have shown that the definition of indicators oncoprotein bcl - 2 and the gene - the p53 tumor suppressor is not only diagnostic value but also has important prognostic and selection of treatment of ovarian dysgerminoma of childhood and adolescence.

Abstract 326

A Preliminary Exploration of a New Risk Stratification System for Childhood Hepatoblastoma

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Purpose: Summarize and analyze the clinical characteristics of pediatric hepatoblastoma and the related factors influencing prognosis, establish a new risk stratification system, and conduct an initial exploration of its prognosis assessment.

Methods: A retrospective analysis was conducted on the clinical data of 388 children with HB from May 2005 to May 2024. A new risk stratification system was established and compared with the previous risk stratification system using the ROC curve.

Results: A new risk stratification system was established, divided into three groups: low, medium, and high risk. There were significant statistical differences in the survival rates; the area under the ROC curve (AUC) was 0.861, which was higher than that of the SIOPEL (0.751) and COG (0.789).

Conclusion: The newly established risk stratification system has a better predictive reference value for the prognosis of children with HB compared to other stratification systems.

Abstract 327

Clinical Experience with Triple Chemotherapy Combined with Induction and Rhythmic Chemotherapy for Children with Neuroblastoma and Central Nervous System Metastasis

YI ZHANG

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Purpose: By summarizing case analyses of children with neuroblastoma (NB) and central nervous system (CNS) metastasis who achieved favorable outcomes through standardized treatment, this study provides a clinical reference for comprehensive diagnosis and treatment of NB with CNS metastasis.

Methods: Three high-risk NB children with CNS metastases were collected as the research subjects. They were treated with a combination of triple sheath injection, chemotherapy , surgery, and radiotherapy, and followed up regularly to observe their comprehensive evaluation indicators.

Results: All three patients were treated with a triple sheath injection (methotrexate+cytarabine+dexamethasone) combined with regular chemotherapy regimen until the end of CR. 3patients were followed up for 21-69 months, respectively, and still EFS.

Conclusion: Combining CNS metastasis with NB is difficult to treat, but choosing a reasonable comprehensive diagnosis, treatment, and follow-up principle is still expected to achieve good remission and long-term survival.

Abstract 328

Clinical Study of Multidisciplinary Diagnosis and Treatment in Children with Soft Tissue Sarcoma Harboring TP53 Mutation

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Purpose: The purpose of this study was to analyze the clinical prognosis of individualized comprehensive diagnosis and treatment for children with TP53-mutated soft tissue sarcoma through multidisciplinary discussion.

Methods: Children admitted with TP53 mutation were collected as the research objects. The MDT diagnosis and treatment plan of Beijing Tongren Hospital was adopted to observe the objective response rate, survival time extension, and secondary surgery, followed by statistical analysis.

Results: 17 children were enrolled, with a median age of 10.1 years.After 4 cycles of comprehensive treatment, evaluation showed that 10 cases achieved. The ORR was 76.5%.The average follow-up time was 31.6 months.The OS was 52.9% (9/17).

Conclusion: Importance should be attached to multidisciplinary diagnosis and treatment, and efforts should be made to seek complete surgical resection and early diagnosis. Standardized initial diagnosis and treatment are crucial for improving the prognosis.

Abstract 329

Clinical Efficacy of Triple Intrathecal Injection Combined with Chemotherapy in Children with Rhabdomyosarcoma Complicated by Meningeal Metastasis

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Purpose: Meningeal metastasis is a major cause of death in children with rhabdomyosarcoma (RMS). This study aimed to evaluate the efficacy and safety of triple intrathecal injection combined with chemotherapy in children with RMS complicated by meningeal metastasis.

Methods: 7 cases of RMS and meningeal metastasis were enrolled and received 4 regular cycles of triple intrathecal injection (methotrexate + cytarabine + dexamethasone) combined with chemotherapy . The remission of meningeal metastasis, therapeutic efficacy, and safety profiles were monitored and analyzed.

Results: 5 cases were alveolar RMS and 2 were embryonal RMS. Leptomeningeal metastasis was confirmed by imaging in all 7 cases, and 4 cases were positive for CSF. After 4 treatment cycles, all patients achieved imaging remission and negative CSF results. As of October 30, 2025, The OS was 100%.

Conclusion: Triple intrathecal injection combined with chemotherapy exhibits favorable clinical efficacy in treating RMS with meningeal metastasis, and the treatment is well-tolerated.

Abstract 330

I Clinical Observation of Entinostat in Children with Alveolar Rhabdomyosarcoma Harboring PAX Family Gene Positivity

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Purpose: Histone deacetylases (HDACs) inhibitors have shown antitumor activity in PAX3-FOXO1-positive alveolar rhabdomyosarcoma (aRMS) mouse models. To investigate the diagnostic and therapeutic value and safety of the HDAC inhibitor entinostat in children with aRMS harboring PAX family gene positivity.

Methods: Pediatric aRMS cases with PAX family gene mutations or fusion positivity were collected between December 2024 and December 2025. A prospective, open-label, controlled study was conducted, with patients divided into a monotherapy maintenance group and a combined chemotherapy group.

Results: A total of 11 children with aRMS were enrolled, including 5 in the monotherapy group and 6 in the combined therapy group.The average medication duration was (4.68±2.17) m. The objective response rate (ORR, CR+PR) was 100.0% in the monotherapy group and 66.7% in the combined therapy group.

Conclusion: Entinostat exerts an inhibitory effect on children with PAX family-positive aRMS with reliable safety, and is expected to be used as a drug to synergistically enhance chemotherapy and maintenance treatment.

Abstract 331

Efficacy and Safety of Dinutuximab Beta Combined with GM-CSF and Isotretinoin ± Chemotherapy as First-line Maintenance Treatment for Pediatric High-risk Neuroblastoma in China

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Purpose: Real-world evidence on dinutuximab beta for high-risk neuroblastoma (NB) in the Chinese pediatric patients remains limited. This study evaluated clinical efficacy and safety of dinutuximab beta as first-line maintenance therapy in a real-world setting.

Methods: We retrospectively analyzed pediatric patients newly diagnosed with HR NB who after induction and consolidation therapy, received dinutuximab β combined with GM-CSF and isotretinoin, with or without chemo. The primary

outcome was ORR; secondary outcomes included 1y and 2y EFS and OS, and safety.

Results: Twenty-eight patients were included. The CR maintenance rate was 78.6%. In patients with PR, the ORR at the EOT was 64.3%. PR patients receiving dinutuximab β with chemo had a higher ORR. The 1y and 2y EFS were 88.2% and 72.4%; 1y and 2y OS were 95.8%. AEs were common but mostly mild to moderate.

Conclusion: Dinutuximab β shows favorable efficacy with HR NB. Patients treated with prior ASCT or combined with chemotherapy showed trends toward improved response rates and survival outcomes. AEs are generally manageable, and the use of standardized pain assessment combined with multimodal analgesia has enabled a substantial reduction in morphine exposure.

Abstract 332

SURGERY AND RISK FACTORS PREDICTIVE OF COMPLICATIONS IN RETROPERITONEAL GERM CELL TUMORS IN CHILDREN

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Purpose: Primary retroperitoneal germ cell tumors (GCT) are rare, accounting for less than 4% of all GCT cases. This study aims to highlight the surgical challenges associated with these tumors and to identify risk factors for perioperative complications.

Methods: We evaluated 33 patients (18 males and 15 females) with retroperitoneal GCT treated between December 2006 and December 2024. The extent of the disease and its relationship to the kidneys and major blood vessels were assessed using CT scans, along with demographic details and surgical complications.

Results: The median age was 16 months. Eighteen patients with benign tumors underwent upfront surgery, while 15 patients with yolk sac tumors received chemotherapy before surgery. The survival rates were 93% for teratomas and 87.5% for malignant GCT.

Conclusion: Surgical outcomes for retroperitoneal GCT are generally favorable; however, extensive vascular dissection and the risks to adjacent structures contribute to a high rate of complications.

Abstract 333

Prognostic factors of hepatocellular carcinoma in children and adolescents: Insights from the Surveillance, Epidemiology, and End Results Database

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Purpose: Hepatocellular carcinoma (HCC) is an uncommon yet highly aggressive cancer in pediatric and adolescent populations. Current understanding of its epidemiological characteristics and survival determinants remains limited. This population-based study aims to evaluate the prognostic factors influencing outcomes in childhood HCC.

Methods: Using a retrospective cohort design, we analyzed data from the SEER registry for HCC cases diagnosed in patients under 20 years between 2000 and 2019. Demographic variables, clinical features, treatment modalities, and survival outcomes were examined. Survival analysis was performed using Kaplan-Meier curves, stratified log-rank tests, and Cox proportional hazards regression models.

Results: The study included 246 pediatric and adolescent HCC patients. The 3-, 5-, and 10-year survival rates were 56.2%, 46.9%, and 38%, respectively, with no notable variations by age, gender, or ethnicity. Improved survival was observed in cases with localized disease and early-stage tumors, particularly among patients undergoing surgical treatment. Multivariable analysis identified SEER stage, T stage, and surgical intervention as independent predictors of overall survival. Notably, liver transplantation demonstrated superior survival benefits compared to partial hepatectomy (HR 0.33, 95% CI 0.16–0.71; P=0.005). A predictive nomogram was developed to estimate individualized survival probabilities, emphasizing the critical role of surgical approaches in optimizing outcomes.

Conclusion: Pediatric HCC is associated with a relatively poor prognosis. Surgical intervention, particularly liver transplantation, appears the most significant factor in improving survival outcomes. The findings also highlight the importance of early diagnosis and accurate staging in managing pediatric HCC.

Abstract 334

Clinical features of mediastinal neuroblastoma in children and development of a nomogram for predicting survival

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Purpose: To identify factors determining the prognosis of primary mediastinal neuroblastoma (NB), we developed a nomogram to predict overall survival (OS) in pediatric patients with mediastinal NB.

Methods: All pediatric patients with mediastinal NB identified between 2000 and 2019 were collected using the Surveillance, Epidemiology, and End Results (SEER) database. To compare survival curves, the log-rank test was used. A multivariate Cox proportional hazards model was developed to investigate the effect of each factor on overall survival. Based on the results of the Cox regression model, a nomogram was constructed.

Results: A total of 192 mediastinal NB patients were identified. Overall survival rates for all patients were 91.0% at 3- year and 89.6% at 5- year, respectively. Age had a substantial impact on survival rates, with younger patients having better outcomes. The outcome of Cox proportional hazard regression revealed that age and surgery were important independent predictors in this model. We developed a nomogram for predicting OS in pediatric mediastinal NB patients based on the Cox regression model. In patients, the risk of death increased with age. Furthermore, patients with no surgery have a lower survival rate than those with surgery.

Conclusion: Our study revealed that age and surgery were found to be the most important predictors of the overall survival of pediatric mediastinal NB, providing critical epidemiological data for clinical study.

Abstract 335

Clinical characteristics and prognostic models of lung tumor in children and adolescents: a population-based study

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Purpose: This study aims to investigate the clinical characteristics, treatment patterns, and prognostic factors associated with primary malignant lung tumors in children and adolescents using data from the SEER database.

Methods: Data from the SEER database between 2000 and 2022 were analyzed, including 370 patients aged ≤19 years diagnosed with primary malignant lung tumors. Demographic, clinical, and treatment data were collected, including histological subtype, tumor stage, treatment modalities, and survival outcomes. Survival analysis was performed using the Kaplan-Meier method, and prognostic factors were assessed using univariate and multivariate Cox regression analyses. A nomogram was constructed to predict 1-, 3-, 5-year OS based on significant predictors.

Results: The most common tumor types were carcinoid tumors (31.6%), pulmonary blastomas (23.8%), and mucinous epithelial carcinoma (12.4%). Surgical resection was the most frequent treatment (78.6%), followed by chemotherapy (35.7%) and radiation therapy (14.9%). The 1-year, 3-year, and 5-year OS rates were 89.5%, 81.2%, and 78.7%, respectively. Histological subtype, SEER stage, and surgical treatment were significant prognostic factors. Carcinoid tumors had the best survival outcomes, while pulmonary blastomas and other rare tumors had poorer survival. Distant-stage disease and chemotherapy were associated with worse outcomes. The nomogram developed based on multivariate analysis provided a useful tool for predicting OS.

Conclusion: Primary malignant lung tumors in children and adolescents exhibit distinct clinical characteristics and survival outcomes based on histology and tumor stage. Early detection and surgical treatment significantly improve outcomes.

Abstract 336

The level of GD2 antigen expression in bone and soft tissue sarcomas in children

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Purpose: The study examines the variations in GD2 expression and its effect on long-term outcomes.

Methods: 52 patients with refractory/relapsing bone and soft tissue sarcomas (STS) were included in the study. In 22 (42%) children STS was diagnosed, in 19 (37%) - osteosarcoma (OS), in 11 (21%) - Ewing sarcoma (ES). The study protocol involved using the BD Medimachine System to break down tumor material and create a suspension of tumor cells. The cells were then treated with antiGD2 antibodies and analyzed using the BD FACSCanto 10-Color Configuration flow cytometer.

Results: The GD2 expression ranged from 1.1 to 95% (mean 39±5%). It should be noted that the average GD2 expression on ES cells was lower than on STS and OS (15±1% vs 60±9% and 21±7%, respectively). No significant correlations (r=0.08; p=0.619) were found in the analysis of the association between the associative GD2 test and progression survival. A cut-off could not be determined using ROC analysis which was conducted on cohorts of patients with different diseases: AUC STS was 0.516, p=0.921, OS AUC was 0.699, p=0.168 and ES AUC was 0.58, p=0.759.

Conclusion: The threshold for GD2 positivity in sarcomas does not have a universally accepted standard. Different studies use different arbitrary values (e.g., 1%, 5%, or 20%) for this threshold, but none of these values has been validated as a reliable predictor of response to treatment. The level of GD2 varies widely among sarcomas, and foci with GD2-positive cells can coexist with areas that are GD2-negative. Biopsies taken from a single location do not necessarily reflect the overall GD2 expression. Moreover, GD2 can change under the influence of treatment, and the tumor microenvironment. Due to these factors, any threshold for GD2 becomes conditional. At present, GD2 remains a research-oriented biomarker, and even weakly positive tumors can respond to treatment directed against GD2.

Abstract 337

The oncological and functional outcomes of children with osteosarcoma after amputation and limb-saving surgery

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Purpose: Despite the growing popularity of limb-saving surgery for bone tumors, there is still a need for in-depth analysis of long-term functional and oncological outcomes in various clinical situations. The purpose is to analyze and compare the outcomes of limb-saving surgery and amputations/disarticulation in children with osteosarcoma

Methods: For 35 (28%) children with osteosarcoma, amputation was performed (group A). Another 88 (72%) patients underwent limb-saving surgery (group B). The patients' characteristics were the same in both groups. A high rate of distant metastases was observed in patients after amputation. The frequency of local relapses was minimal, and the same for both study groups.

Results: The overall three-year survival in group B was higher than that of group A (69 ± 5% vs. 34 ± 8%, p<0.0001). Significant differences were found in relapse-, event-, and progression-free survivals: 57 ± 6% vs 24 ± 7%, p = 0.0012; 50 ± 6% vs 26 ± 8%, p = 0.0031 and 62 ± 6% vs 42 ± 9%, p = 0.0047, respectively. In group A soft tissue failures were diagnosed in 6 patients (6.8%), aseptic instabilities in 4 (5%), prosthesis component fractures in one (2%), and infections in 9 (10%). After amputations complications included soft tissue failure in 14 (11%) patients, tumor progression in 2 (6%), and infection in one patient (3%). In group B, MSTS scores (functional outcomes) were higher (21.83 ± 3.84) than those of group A (6.68 ± 1.18) (p < 0.5).

Conclusion: Reconstructive surgery is the preferred treatment for malignant limb tumors in children. Children with osteosarcoma have favorable prognoses in terms of cancer safety and controlled complications. Endoprosthesis can be a valuable option for restoring limb function in younger children.

Abstract 338

Pediatric Hepatoblastoma: Surgical and Chemotherapy Approaches Tashkent State Medical University, Pediatric Hematology Center, Oncology and Clinical Immunology Center

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Purpose: Hepatoblastoma is the most common primary liver malignancy in children. Effective management requires a multidisciplinary approach that combines surgical intervention with chemotherapy. Surgical resection remains the cornerstone of treatment, aiming for complete tumor removal while preserving functional liver tissue. Neoadjuvant chemotherapy plays a critical role in reducing tumor size, enabling resection of initially unresectable tumors, and targeting micrometastases. Adjuvant chemotherapy following surgery eliminates residual tumor cells, decreases recurrence risk, and improves long-term survival.

Methods: Review of clinical data, modern surgical techniques (partial hepatectomy, liver transplantation), and chemotherapy regimens (cisplatin, doxorubicin, vincristine).

Results: The integration of surgery and chemotherapy significantly improves five-year survival rates. Neoadjuvant therapy facilitates complete resection in previously inoperable cases, while adjuvant therapy ensures long-term disease control. Treatment strategies tailored to tumor stage, histology, and patient condition optimize outcomes and minimize complications.

Conclusion: A multidisciplinary approach combining surgical expertise and optimized chemotherapy protocols represents the most effective strategy for managing pediatric hepatoblastoma. Early diagnosis, timely surgical intervention, and individualized chemotherapy regimens substantially enhance prognosis, reduce recurrence, and improve quality of life in affected children.

Abstract 339

The role of intrathecal chemotherapy in the treatment of children with rhabdomyosarcoma of parameningeal localization with intracranial spread and/or leptomeningeal metastasis

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Purpose: Rhabdomyosarcoma (RMS) of parameningeal localization with intracranial spread is characterized by an aggressive course and a high risk of leptomeningeal metastasis (LM). The blood-brain barrier (BBB) limits the effectiveness of rhabdomyosarcoma therapy in children with intracranial spread, limiting the penetration of chemotherapy drugs into the central nervous system (CNS), which makes the search for methods to control leptomeningeal metastasis one of the key tasks of pediatric oncology. Aim. To evaluate the efficacy and safety of intrathecal chemotherapy (ITHT) in the complex treatment of children with parameningeal RMS and CNS lesions, as well as to improve survival rates in this group.

Methods: The study included 21 patients with verified parameningeal RMS with intracranial spread and/or leptomeningeal metastasis who were treated at the Institute of Pediatric Oncology and Hematology. Academician of the Russian Academy of Medical Sciences L.A. Durnov in the period from 2021 to 2024.

Results: Median follow-up was 34.3 months; overall 2—year survival (S) was 65%. The effectiveness of first-line ITHT (preventive and therapeutic) reached 80% (2-year period), which is significantly higher than in the relapse group (20%, p < 0.05). The obtained indicators significantly exceed the foreign data (median survival rate of 4-6 months in LM). The toxicity profile of ITHT is favorable, no severe neurotoxicity has been recorded.

Conclusion: The use of intensive IPT in the complex therapy of children with parameningeal RMS and/or CNS is a safe and highly effective method that significantly improves survival in this extremely unfavorable group.

Abstract 340

The diagnostic reasoning for pediatric rhabdomyosarcoma presenting with bone marrow metastasis

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Purpose: Rhabdomyosarcoma (RMS) constitutes the most prevalent soft tissue sarcoma encountered in the pediatric and adolescent population, yet bone marrow metastasis represents a relatively infrequent clinical manifestation. RMS with concurrent bone marrow infiltration typically exhibits atypical clinical symptomatology and bone marrow morphological characteristics, which frequently leads to initial misdiagnosis as hematological malignancies or other solid neoplasms, ultimately precipitating therapeutic delay.

Methods: We performed a diagnostic analysis on an 11-year-old male patient whose initial symptoms were a hemorrhagic rash and arthralgia.

Results: Preliminary bone marrow cytomorphological analysis raised a provisional diagnosis of acute myeloid leukemia (AML). Subsequent immunophenotypic profiling was insufficient to exclude neuroblastoma (NB). Definitive diagnosis of alveolar rhabdomyosarcoma (ARMS) was established via contrast-enhanced magnetic resonance imaging (MRI), positron emission tomography-computed tomography (PET-CT) and histopathological biopsy combined with immunohistochemical validation.

Conclusion: This clinical case demonstrates that RMS can manifest initially with bone marrow metastasis, mimicking the clinical and laboratory features of hematological malignancies. RMS should be incorporated into the differential diagnostic spectrum when blast cells are detected in bone marrow smears. Definitive etiological diagnosis necessitates a comprehensive diagnostic strategy encompassing immunohistochemical biomarker detection, molecular genetic testing, and advanced imaging modalities for primary tumor localization.

Abstract 341

A Novel Lactylation-Based Prognostic Model for Neuroblastoma: Biomarker Identification and Independent Cohort Validation

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Purpose: Neuroblastoma (NB) is a prevalent malignant tumor in children, particularly characterized by a high recurrence rate among high-risk patients.Lactylation plays a critical role in regulating metabolic and immune processes in tumors. The objective of this study was to identify key lactylation genes in NB and construct a robust prognostic model to stratify NB patients based on survival outcomes.

Methods: This study analyzed transcriptomic and clinical data from 498 NB patients sourced from the GEO database. A subset of 332 lactylation genes was evaluated using weighted gene co-expression network analysis (WGCNA) and machine learning algorithms, including Random Forest and eXtreme Gradient Boosting (XGBoost), to identify differentially expressed genes. Subsequent analysis of these genes was performed to develop a prognostic model.

Results: Our findings revealed two distinct molecular clusters of NB, characterized by significant variations in tumor microenvironment and survival outcomes. By leveraging the differentially expressed lactylation genes CCNA2 and TP53, we successfully developed a prognostic model that stratified patients into high-risk and low-risk categories, demonstrating robust predictive performance validated in an independent cohort.

Conclusion: This study underscores the potential of lactylation gene expression as a valuable prognostic biomarker in NB, paving the way for personalized treatment strategies. Future research should focus on the functional characterization of these genes to further elucidate their role in NB pathogenesis and progression.

Abstract 342

APPROACHES TO THE TREATMENT OF RHABDOMYOSARCOMA IN CHILDREN.

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Purpose: Rhabdomyosarcoma is the most common mesenchymal malignant tumor in children. According to statistics in the Republic of Uzbekistan, rhabdomyosarcoma is 3-5% of the total incidence and is included in the group of relatively rare diseases. Purpose: To study the influence of the molecular and genetic characteristics of rhabdomyosarcoma while choosing treatment tactics and determining prognostic aspects in conditions of Practical Medical Center of Pediatric Hematology, Oncology and Immunology of the Ministry of Health of the Republic of Uzbekistan.

Methods: the study included 45 pediatric patients with rhabdomyosarcoma registered in 2023-2025. The tumor was localized in the corpus in 8 patients (17.8%), in the extremities in 10 (22.2%), in the perineum in 4 (8.9%), in the head and neck in 15 (33.3%), in the bladder in 5 (11.1%), and in the pelvis in 3 (6.7%). Histological type of tumor: embryonal – 29 (64,4%), alveolar – 16 (35,6%). For alveolar rhabdomyosarcoma: FOXO1 gene translocations: positive – 10 (62.5%), negative – 6 (37.5%). The average age of the patients was 5.31 years. All patients were diagnosed with different stages of the disease. At the first step, all patients after histological verification, underwent neoadjuvant polychemotherapy from 2 courses to 6 courses (PCT) according to the VAIA III CWS 2012 and IRS Regimen B.

Results: Complete tumor regression was observed in 3 (6,67%), partial regression - in 28 (62,2%), in 11(24,4%) without effect, in 3(6,67%) progression of the disease. Polychemotherapy courses were carried out with concomitant therapy. At the second stage, surgical interventions and for patients over 5 years old additionally radiation therapy were performed. In the postoperative period, up to 6 courses of adjuvant polychemotherapy were carried out. Three-year relapse-free and metastasis-free survival was 65.4%.

Conclusion: A genetic-based approach to treatment of rhabdomyosarcomas in children significantly improves outcomes and increases relapse-free and metastasis-free survival of patients.

Abstract 343

Rare case of MutYH-associated polyposis in a 12-year-old-child

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Purpose: MutYH-associated polyposis (MAP) is a rare autosomal recessive cancer predisposition syndrome caused by pathogenic variants in the MUTYH gene. While typically linked to colorectal cancer, MAP may present with extracolonic malignancies. Pediatric cases are uncommon, and early recognition remains challenging.

Methods: We report a 12-year-old girl presenting with fatigue and severe anemia (hemoglobin 63 g/L), unresponsive to iron therapy. Further evaluation revealed a duodenal mass on imaging and endoscopy, with high metabolic activity on 18 FDG PET-CT (SUVmax 26.0). Colonoscopy demonstrated multiple polyps (2–3 cm), and thyroid ultrasound identified bilateral nodules. Histopathology confirmed duodenal adenocarcinoma. The patient underwent pancreaticoduodenectomy with lymphadenectomy; staging showed intestinal-type adenocarcinoma, pT3N0M0, KRAS-mutant (p.G12C), microsatellite stable. Given the unusual presentation, genetic testing was performed and revealed biallelic MUTYH mutations (exome 7 and 12), establishing the diagnosis of MAP. The patient received adjuvant capecitabine for six months. Thyroidectomy was performed due to suspicious nodules; histology showed multiple follicular adenomas. During follow-up in 2 years, progression to extensive colonic polyposis required subtotal colectomy that we discussed with both the patient and her parents. Six months later a subsequent sigmoid lesion was resected endoscopically and identified as adenocarcinoma in situ (T1N0M0, stage I), not requiring further treatment.

Results: At latest follow-up, the patient is in good clinical condition with no evidence of advanced disease. Surveillance includes regular endoscopic and radiologic assessments.

Conclusion: This case represents an exceptionally early and aggressive pediatric presentation of MAP with duodenal adenocarcinoma, a manifestation reported only in limited adult cases and rarely described as an initial presentation. It highlights three key points: (1) extracolonic malignancies may be the first manifestation of MAP in children; (2) early comprehensive genetic testing is

critical in atypical or multiple tumors; and (3) surveillance strategies must be individualized and intensified. Increased awareness of rare tumor presentations can enable timely diagnosis and improve outcomes in pediatric cancer predisposition syndromes.

Abstract 344

TANDEM AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION IS SAFE AND EFFECTIVE FOR TREATMENT OF PEDIATRIC GERM CELL TUMORS: SINGLE CENTER EXPERIENCE FOR 18 CHILDREN

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Purpose: Tandem autologous hematopoietic stem cell transplantation (aHSCT) is an option for high risk germ-cell tumors (GCT) in children. Safety profile and efficacy of tandem aHSCT requires to be investigated for GCT.

Methods: This is a retrospective analysis of tandem aHSCT for 20 pediatric patients with GCT in Nikolay Blokhin National Medical Research Center of Oncology in 2020-2026. We aimed to define the type and cumulative incidence of complications after aHSCT for pediatric GCT, as well as the overall outcomes.

Results: Data were received for 20 children (median of age 2 years, range 11 months-17.6 years), who received tandem aHSCT for GCT between 2020 and 2026 in Nikolay Blokhin National Medical Research Center of Oncology. M:F=8:12. Median follow-up was 18 months (2.5-62). Conditioning regimen: first aHSCT: Eto+Carbo, second – Eto+Thio. Median of CD34+ cell dose was 4,58×10 /kg (3.3 – 9.4) and 4,22×10 /kg (3.1 – 8.4) for the first and second aHSCT retrospectively. There were no significant correlation between the number of CD34+ cells and the day of ANC recovery. Stem cell source: PBSC – 100%. Median of ANC recovery was 11.8 (10.5-14.7) and 12.4 (10.2-15.6) days for first and second aHSCT retrospectively. At 3 months, the cumulative incidence for transplant related toxicities (infections, hepatic and nephrotoxicity) of 1-2 gr. was 87.3% (95% Confidence Interval (CI): 65.3-90.4), 3-4 gr. was only 6.3% (95% CI: 5.8-12.9). Only 2 patients died: 1 – relapse, 1 – late infectious complications. Eighteen patients alive in complete remission.

Conclusion: Our study confirms that tandem aHSCT for a pediatric GCT is safe and effective option. Transplant-related toxicity (both infectious and noninfectious) is acceptable. NRM is comparable low. Future multicenter studies required to improve the results of treatment of high risk GCT in children.

Abstract 345

High-dose polychemotherapy with autologous peripheral stem cell transplantation in hepatoblastoma. Experience of two centres.

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Purpose: Hematopoietic stem cell transplantation (HSCT) continues to be a feasible treatment option for a number of malignant and non-malignant diseases. However, in some cases the indications for this method are not so evident due to the rarity of the condition in question. Liver tumours account for 1-2% of all malignant conditions in children. Despite the fact that modern treatment regimens can cure more than 80% of patients, there are still some prognostic ally unfavorable cohorts, primarily patients with metastatic lesion, inoperable chemo-resistant tumors, and the disease recurrence. In these cases the possible role of intensive consolidation may be debated.

Methods: Clinical case No. 1 A 2-year-old patient diagnosed with left liver lobe hepatoblastoma T3N0M1 PRETEXT III St. IV (left lung metastases), high-risk group. The patient was treated by neoadjuvant chemotherapy with subsequent surgery and adjuvant chemotherapy according to GPOH HB99 protocol for high-risk group achieving complete response. Due to initial disease dissemination he then received high-dose consolidation (Carboplatin, Etoposide, Melphalan) with autologous HSCT. Clinical case No. 2 A 14-year-old patient with right liver lobe diagnosed hepatoblastoma PRETEXT 3, second early local relapse. The initial therapy was conducted according to SIOPEL 3 protocol for high-risk group (neoadjuvant chemotherapy, hemihepatectomy, adjuvant chemotherapy) leading to complete response. Then, 3 months later, a 1st local relapse developed and was treated according to SIOPEL guidelines with 2 courses of 2nd-line chemotherapy (vincristine, irinotecan), atypical liver resection, and 9 courses of adjuvant chemotherapy. Although subsequent imaging showed no signs of residual disease, the alpha-fetoprotein values were still slightly elevated (AFP; 33.2 IU/mL). After 7 months of follow-up a second local recurrence was confirmed. As after tumor resection the AFP level still remained high, a tandem high-dose chemotherapy (etoposide 2000 mg/m2 for 3 days, carboplatin 2000 mg/m2 for 3 days with a 28-day interval) with auto-HSCT (5.5x106 and 2.1x106 CD34 +/kg) was performed.

Results: Clinical case No. 1 The early post-transplant period was complicated by Gr. 2-3 oropharyngeal and gastrointestinal mucositis, Gr. 2 hepatotoxicity, Gr. 3 infectious complications. Engraftment was registered on Day + 13 and the patient retains complete response during 7-year follow-up period. Clinical case No. 2 Engraftment was registered on days + 10 and + 11, respectively. The toxicity of both regimens was acceptable. After 1.5 years of follow-up, the patient experienced a relapse of the underlying disease.

Conclusion: Dose-intensive consolidation is potentially effective in selected narrow subgroups of patients. Nevertheless, obtaining reliable information and forming a general approach to the use of this method is possible only within multi-centre cooperative groups.

Abstract 346

Robot-assisted resection of adrenal tumor in infants less than 6 months of age

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Purpose: Robotic assisted surgery (RAS) has been widely used in pediatric patients, but its application in infants with adrenal tumors is rarely reported. The aim of this study is to evaluate the feasibility and safety of using RAS for adrenal tumor in children less than 6 months of age, and discuss the key technical points and skills.

Methods: We reviewed the consecutive RAS procedures in infants with adrenal tumor between January 2020 and December 2025. The inclusion criteria enrolled in this study was localized tumor confined to one body compartment. Patients who undergone emergency surgery, had abdominal surgery history, or had any pre-existing health conditions that would preclude safe initiation or maintenance of operation were excluded. Patient demographics, operative details, postoperative outcomes, and follow-up were recorded.

Results: A total of 28 patients were enrolled in this study, including 23 with neuroblastoma, 3 with teratoma, and 2 with adrenal cortical tumors. The median age was 69.87 days (interquartile range [IQR], 14–105), the median body weight was 5.35 kg (IQR, 3.5–6.7), and the median maximum tumor diameter was 42.37 mm (IQR, 31–51). The median total operative time was 96.57 minutes (IQR, 67–122), the median intraoperative blood loss was 10.65 mL (IQR, 2–50), and the median postoperative hospital stay was 5.17 days (IQR, 3–6). Intraoperatively, one case was converted to open laparotomy due to inferior vena cava injury. One neuroblastoma patient developed tumor spillage, and no other major perioperative complications were observed in the remaining patients. This aforementioned patient with tumor rupture spillage developed tumor recurrence at both the primary tumor site and the trocar incision site, and is currently receiving systemic chemotherapy. No mortality was recorded during the follow-up period.

Conclusion: Robot-assisted resection for localized adrenal tumor in infants less than 6 months of age appears technically feasible and safe. In the event of tumor spillage, prompt conversion to open laparotomy should be performed.

Abstract 347

Laparoscopic Wilms Tumor Nephrectomy in Children: Technical considerations and short term outcomes

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Purpose: This single-centre ambispective study included 19 pediatric WT patients undergoing attempted LWTN after neoadjuvant chemotherapy. Data collected covered demographics, surgical details, conversion rates, complications, blood loss, recovery, pain, time to adjuvant therapy, hospital stay, parental satisfaction, margin status, lymph node yield, and tumor rupture. Outcomes of successful LWTN were compared with those that required conversion.

Methods: This single-centre ambispective study included 19 pediatric WT patients undergoing attempted LWTN after neoadjuvant chemotherapy. Data collected covered demographics, surgical details, conversion rates, complications, blood loss, recovery, pain, time to adjuvant therapy, hospital stay, parental satisfaction, margin status, lymph node yield, and tumor rupture. Outcomes of successful LWTN were compared with those that required conversion.

Results: Results: LWTN was initiated in all 19 cases, with 63.1% successfully completed laparoscopically. Conversion to open surgery was necessary in 36.8% of patients due to intraoperative bleeding, adhesions, or technical challenges. The LWTN group demonstrated significantly reduced intraoperative blood loss (median 20 ml vs. 40 ml; $p = 0.014$), earlier initiation of adjuvant chemotherapy (1.63 vs. 2.56 weeks; $p < 0.001$), shorter hospital stays (3.13 vs. 4.11 days; $p = 0.023$), lower postoperative pain scores ($p = 0.001$), and greater parental satisfaction ($p < 0.001$) compared to the conversion group. Both groups achieved comparable R0 resection and lymph node yields that met accepted oncologic standards; however, tumor rupture occurred in two patients.

Conclusion: LWTN was feasible and safe in selected cases, providing perioperative benefits without compromising surgical or oncologic outcomes. Cautious use by experienced teams is recommended, with strict adherence to selection criteria and oncologic guidelines.

Abstract 348

NON-PARAMENINGEAL RHABDOMYOSARCOMA: CONFRONTING THE CRITICAL CHALLENGES OF RESECTION AND RECONSTRUCTION

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Purpose: Addressing non-parameningeal rhabdomyosarcoma (RMS) poses significant challenges in both resection and reconstruction. Overcoming these obstacles is crucial for improving patient outcomes and enhancing quality of life. This study aimed to describe the surgical treatment of localized non-parameningeal RMS in children.

Methods: We evaluated all patients with primary RMS located in non-parameningeal sites who underwent multimodal treatment, consisting of chemotherapy, surgery, and/or irradiation, from 2006 to 2024.

Results: The primary sites of RMS were distributed as follows: face (57.6%), oral cavity (24.2%), neck (12.1%), and salivary glands (6.1%). A majority of patients (85%) underwent delayed surgery, while three patients had re-excisions before treatment due to initial incomplete surgery. Complete surgical excision was achieved in 88% of cases; however, four patients had positive microscopic margins. Reconstruction was necessary for 57.5% of the patients, utilizing either local flaps (58%) or free microvascular flaps (37%). The local flaps included forehead flaps, nasolabial flaps, and local rotation flaps, while the free microvascular flaps comprised anterolateral thigh flaps, free fibula flaps, and free radial artery-based flaps. Surgical complications occurred in 15% of patients; however, only two patients experienced Clavien-Dindo grade 3 complications. With a mean follow-up of 65 months, the 5-year event-free and overall survival rates were 69% and 83%, respectively.

Conclusion: Surgical resection of non-parameningeal RMS, although technically challenging and constrained by the need for wide margins, is feasible with acceptable levels of morbidity. A variety of reconstruction procedures are available to effectively address the surgical defects.

Abstract 349

Assessment of the quality of lung lesions diagnosis in adolescents with osteosarcoma using artificial intelligence

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Purpose: The presence of metastases in the lungs in patients with osteosarcoma is the main cause of disease-related death. The accuracy of the diagnosis of pathological nodules by lung CT is critical for the timely diagnosis of the disseminated stage of the disease and the planning of surgical treatment of metastases. In the current study, the working hypothesis is accepted that the use of artificial intelligence in the search for pathological lesions in the lungs can help to increase the diagnostic accuracy and reduce the chance of missing metastases. Aim: to evaluate the quality of lung lesion diagnosis in adolescents with osteosarcoma using artificial intelligence.

Methods: The study conducted a retrospective comparative assessment of computed tomograms (CT) of lungs of patients of Dmitry Rogachev National medical research center of pediatric hematology, oncology and immunology (further – the Center) with osteosarcoma (OS) by the radiologist of the Radiology department of the Center, the artificial intelligence algorithm (AI) of the IRA Labs, the expert of the IRA Labs and (in case of disagreement of opinions) – the radiologist of Research and practical clinical center for diagnostics and telemedicine technologies. The target finding is accepted as a pathological lesion of average size ≥ 4 mm. Based on the analysis performed, the diagnostic accuracy of the AI algorithm was evaluated, the number of false positives (FP) and false negatives (FN) was determined, and sensitivity, specificity, accuracy, area under the ROC curve (AUC), positive predictive value (PPV), negative predictive value (NPV), and F1-measure were calculated. The algorithm's performance was evaluated in accordance with the guidelines for conducting clinical trials of software based on intelligent technologies.

Results: The study included 248 patients with OS aged 13-20 years 11 months 29 days. The evaluation of 248 CT scans of the lungs yielded the following results: in 5 cases, the AI algorithm showed FP result (2.02%), in 34 cases, FN result (13.71%), and in 209 cases, it correctly identified the target pathology (84.27%). The diagnostic accuracy of the algorithm was 0.843 (95% CI 0.794-0.887), and the algorithm's performance was considered satisfactory. By integrating the AI algorithm into a specific clinical task of detecting pathological lesions in the lungs of patients with OS, the sensitivity could be increased from 0.805 to 0.891, resulting in 8.59% absolute reduction in the number of FN results and in 44% relative reduction.

Conclusion: The obtained results objectively confirm the practical value of using the AI algorithm and justify the implementation of AI-assisted systems in the diagnostic protocols for lung metastases in adolescent and young adult patients with OS.

Abstract 350

Disparities in the Surgical Management of Ovarian Malignant Germ Cell Tumors found in children and adolescents: A multicentre retrospective study in China

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Purpose: Paediatric ovarian malignant germ cell tumors (POMGCTs) account for 75–80% of paediatric ovarian malignancies, with fertility preservation as a core clinical priority alongside oncological treatment. In China, the lack of dedicated paediatric gynecologic oncology subspecialties leads to POMGCTs being managed by either paediatric surgeons or gynaecologists, resulting in potential practice variations. Despite established Children's Oncology Group (COG) guidelines, adherence to comprehensive staging surgery remains suboptimal in clinical practice, and data on temporal trends in POMGCT management and independent risk factors for guideline non-adherence in China are scarce. This study aims to address these gaps to inform standardized POMGCT care.

Methods: A multicentre retrospective study was conducted on 102 patients aged ≤18 years with pathologically confirmed POMGCTs, who received primary surgical treatment at 7 tertiary medical centers in China from January 2010 to December 2024. Patients were stratified into Group A (managed by paediatric surgeons, n=86) and Group B (managed by gynaecologists, n=16). Standardized data on demographic characteristics, preoperative diagnostic workup, surgical details, pathological features, adjuvant therapy and follow-up outcomes were collected. Comprehensive staging surgery was defined per COG guidelines. Statistical analyses were performed using SPSS 26.0 and R 4.0.0, including descriptive statistics, intergroup comparisons, Kaplan-Meier survival analysis, linear regression for temporal trend evaluation, and multivariate Logistic regression to identify independent risk factors for guideline non-adherence.

Results: Group A had a significantly younger median age at diagnosis (9 vs 16 years, P<0.001), higher rates of emergency admission (32.6% vs 12.5%, P=0.042) and emergency surgery (18.6% vs 6.25%, P=0.038) compared with Group B. Group B exhibited a higher rate of complete preoperative tumor marker testing (AFP+β-HCG+LDH, 87.5% vs 64.0%, P=0.029), a significantly higher completion rate of COG-concordant comprehensive staging surgery (81.25% vs 37.2%, P<0.001), lower intraoperative tumor rupture rate (6.25% vs 23.3%, P=0.045), and higher fertility preservation rate (87.5% vs 65.1%, P=0.028). Group A had a significantly higher tumor recurrence rate (20.9% vs 6.25%, P=0.036) and lower recurrence-free survival rate (79.1% vs 93.75%, P=0.036), with no significant difference in overall survival between the two groups (90.7% vs 93.75%, P=0.671). Temporal trend analysis across three 5-year intervals (2010–2014, 2015–2019, 2020–2024) showed slight but non-significant improvements in comprehensive staging surgery completion rate, laparoscopic surgery utilization, complete tumor marker testing rate, and tumor recurrence rate (all P>0.05). Multivariate Logistic regression identified paediatric surgical specialty (OR=5.26, 95%CI:1.78–15.57, P=0.003), emergency surgery (OR=3.89, 95%CI:1.42–10.68, P=0.008), and incomplete preoperative tumor marker testing (OR=3.12, 95%CI:1.15–8.47, P=0.026) as independent risk factors for non-adherence to COG staging guidelines.

Conclusion: Significant practice variations exist in the surgical management of POMGCTs between paediatric surgeons and gynaecologists in China, with paediatric surgeon-managed patients showing lower guideline adherence, higher tumor recurrence rates, and poorer fertility preservation outcomes. No meaningful temporal improvement in clinical practice was observed over the 15-year study period. Targeted interventions are urgently needed, including specialized POMGCT surgical training for paediatric surgeons, establishment of standardized preoperative assessment pathways, promotion of multidisciplinary team (MDT) care, implementation of COG-aligned surgical checklists, and institutional quality feedback mechanisms. Integration of paediatric surgical and gynecologic oncology expertise through MDT collaboration is the cornerstone of optimizing POMGCT management, balancing oncological radicality and fertility preservation.

Abstract 351

Should Renal Vessel Contact Be Considered an Image-Defined Risk Factor in Abdominal Neuroblastoma?

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Purpose: Renal vessel contact is classified as an image-defined risk factor (IDRF) in neuroblastoma, implying increased surgical risk and influencing staging and treatment. We evaluated its impact on renal outcomes, risk classification, and potential treatment intensification.

Methods: We retrospectively reviewed children with abdominal neuroblastoma and radiologic renal vessel contact treated at Beijing Children's Hospital between January 2018 and June 2023. Staging and risk stratification were assessed using the 2009 International Neuroblastoma Risk Group (INRG) classification and the 2021 revised Children's Oncology Group (COG) risk classification system.

Results: Fifty-nine patients were included; 44 (74.6%) had renal vessel contact as the sole IDRF and 15 (25.4%) had additional IDRFs. Excluding renal vessel contact as an IDRF reclassified 18 patients. Under the 2009 INRG system, 3 patients were reclassified from intermediate risk to very low risk and 15 from low risk to very low risk. Under the 2021 COG system, 15 patients were reclassified from intermediate risk to low risk and 3 from high risk to low risk. Transient renal ischemia occurred in 1 patient and resolved intraoperatively. No patient underwent nephrectomy, and no renal atrophy was identified at a median follow-up of 43 months. Three-year overall survival, event-free survival, and cumulative incidence of local progression were 89.1% (95% CI, 81.1% to 97.8%), 86.0% (95% CI, 77.5% to 95.5%), and 5.4% (95% CI, 0 to 11.1%), respectively. No oncologic events occurred among the 18 reclassified patients.

Conclusion: Renal vessel contact was not associated with kidney loss but substantially altered staging and risk classification. These findings support reconsideration of renal vessel contact alone as an IDRF in abdominal neuroblastoma.

Abstract 352

Clinicopathological Features of Nephroblastoma in Mongolia

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Purpose: Nephroblastoma (Wilms' tumor) accounts for most pediatric kidney tumors globally. However, there is limited detailed information on the clinicopathological features of Mongolian pediatric patients. This study aimed to evaluate clinicopathological features of nephroblastoma diagnosed in Mongolia.

Methods: This study was based on pathology records from the National Center of Pathology, comprising 14 surgical specimens with a confirmed diagnosis of nephroblastoma collected between 2021 and 2025.

Results: A total of 14 patients were included in the final analysis (one case was excluded). The cohort comprised 8 males (57.1%) and 6 females (42.9%). Mean age was 42.6 ± 27.9 (7–96) months. Mean tumor size was 7.54 ± 3.70 (2.5–16.0) cm. The majority of tumors were unifocal (10/14, 71.4%), while 4 cases (28.6%) were multifocal. Pathological staging revealed Stage I in 5 cases (35.7%), Stage II in 8 (57.1%), and Stage III in 1 (7.1%). Triphasic histology was the most common subtype (7/14, 50.0%), followed by monophasic (4/14, 28.6%) and biphasic (3/14, 21.4%) patterns. Anaplasia was present in 3 cases (21.4%). Nephrogenic rests were identified in 2 cases (14.3%). Risk stratification classified 5 tumors as low risk (35.7%), 7 as intermediate risk (50.0%), and 2 as high risk (14.3%). Renal sinus invasion and positive surgical margins were each observed in 5 cases (35.7%). WT1 immunohistochemistry was performed in 6 cases, all of which were positive.

Conclusion: Nephroblastoma most commonly presents with triphasic histology at intermediate risk; however, the considerable rates of positive surgical margins and renal sinus invasion emphasize the need for rigorous surgical staging and multidisciplinary management. Keywords: Wilms' tumor, pediatric renal neoplasm, risk, histological features

Abstract 353

To summarize and analyze the clinical characteristics of neonatal adrenal masses and to explore their diagnosis and treatment methods

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Purpose: To summarize and analyze the clinical characteristics of neonatal adrenal masses and to explore their diagnosis and treatment methods.

Methods: A retrospective analysis was conducted on the clinical data of 18 pediatric patients with adrenal masses admitted to the First Department of General Surgery (Neonatal Surgery) of Hebei Children's Hospital from June 2018 to June 2025. Patients were divided into Group A (solid tumor group) and Group B (non-solid tumor group) based on the nature of the mass. Collected data included gender, age, tumor location, treatment method (conservative or surgical), and postoperative pathological findings.

Results: A total of 18 pediatric patients with adrenal masses were included, all diagnosed within 28 days after birth, with 11 cases in Group A and 7 cases in Group B. In Group A, preoperative ultrasound and CT scans of all 11 children indicated adrenal neuroblastoma. The mass was detected during prenatal

examination in 10 cases, and in 1 case, it was found during preoperative examination upon admission for congenital hypertrophic pyloric stenosis. The group consisted of 6 males (54.5%) and 5 females (45.5%); the mass was located on the left in 7 cases (63.6%), on the right in 3 cases (27.3%), and bilaterally in 1 case (9.1%). All patients in this group underwent surgical resection. The age at surgery ranged from 6 days to 69 days, with a median surgical age of 21 days. Complete laparoscopic adrenalectomy was performed in 4 cases. In 5 cases, conversion to open surgery was necessary due to the small patient age, limited abdominal space, and difficult laparoscopic manipulation. One patient with concurrent congenital hypertrophic pyloric stenosis underwent direct open surgery. One patient with bilateral adrenal masses on preoperative examination underwent direct open surgery; intraoperatively, considering the size, location, and to avoid postoperative adrenal insufficiency, only the right adrenal mass was resected, and the left mass was left untreated. Postoperative pathological examination confirmed adrenal neuroblastoma in all 11 Group A patients: 10 with favorable histology and 1 with unfavorable histology. According to INSS staging, there were 4 cases (36.4%) in Stage I, 5 cases (45.5%) in Stage II, and 2 cases (18.1%) in Stage IVS. As of the follow-up cut-off in August 2025, no recurrences were observed in this group. In Group B, admission ultrasound suggested neonatal adrenal hemorrhage. The condition was detected during prenatal examination in 3 cases, and discovered during routine abdominal ultrasound upon admission for other concurrent diseases in 4 cases. The group included 5 males (71.4%) and 2 females (28.6%); the hematoma was located on the left in 1 case (14.3%) and on the right in 6 cases (85.7%). Age at admission ranged from 41 hours to 15 days. All patients in this group received conservative treatment including symptomatic hemostasis and regular ultrasound follow-up, and were discharged successfully. The hematomas resolved after 3-6 months of follow-up in this group.

Conclusion: The most common causes of neonatal adrenal masses are adrenal hemorrhage and neuroblastoma. Other rare causes include adrenal cysts, congenital adrenal hyperplasia, and benign tumors. Neonatal adrenal neuroblastoma is often detected during prenatal examinations. While spontaneous regression is possible in a very small subset of patients, surgical resection remains the primary treatment modality. Postoperative chemotherapy is not required for Stage I and II disease but is necessary as adjuvant therapy for advanced stages (III, IV) and progressive Stage IVS disease. Neonatal adrenal hemorrhage is frequently caused by birth trauma, hypoxia, or coagulation disorders, typically presenting as swelling or hematoma, predominantly on the right side. Satisfactory outcomes can be achieved with conservative management.

Abstract 354

Analysis of primary hepatic tumors in the children treated at a tertiary care center in LMIC

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Purpose: Pediatric hepatic tumors are rare, with hepatoblastoma (HB) being the predominant subtype. While international studies have identified prognostic factors such as age, AFP, and PRETEXT stage, large scale systematic analyses from India have been lacking.

Methods: This was a single center, retrospective observational cohort study conducted at Tata Memorial Hospital, Mumbai, between February 2006 and December 2024. A total of 505 children ≤15 years with primary hepatic tumors were registered, of which 423 primary cases were included in the analysis (HB: 340, non HB malignant: 43, non HB benign: 40). Clinical data of primary hepatic tumors including demographics, AFP levels, and PRETEXT/POSTTEXT staging were extracted from electronic records. Survival outcomes were analyzed using Kaplan–Meier curves with log rank testing. Cox regression was performed to identify independent prognostic factors (age, AFP, PRETEXT stage, risk stratification, and surgery).

Results: A total of 505 children with primary hepatic tumors were registered, of which 423 primary cases were analyzed (HB: 340, non HB malignant: 43, non HB benign: 40). Children with hepatoblastoma presented at a median age of 19 months (IQR 29.75). In contrast, those with non HB malignant tumors were significantly older, with a median age of 117 months (IQR 101), while patients with non HB benign tumors had a median age of 14.5 months (IQR 54). Stratified by subtype, HB patients had a median follow up of 38 months (IQR 17–70), Survival analysis showed that within the HB cohort (n=340), the 5 year EFS was 64.2% and OS 68.4%. Outcomes improved in the TMH only treated HB group (n=274), with 5 year EFS 70.7% and OS 74.6%, and were best in the surgery centric cohort (n=202), achieving 5 year EFS 86.4% and OS 89.8%. Non HB malignant tumors with median follow up of 36 months (IQR 15–68), and non HB benign tumors with 44 months (IQR 20–75). Non HB malignant tumors had a 3 year OS of 36.1% in the whole cohort, improving to 45.1% in TMH only treated patients, while benign tumors demonstrated excellent survival with 5 year OS/EFS of 89.5–100%. Univariate analysis in HB demonstrated that younger age (≤4 years) was associated with superior OS ($p<0.001$) and EFS ($p<0.001$), while advancing PRETEXT stage correlated with progressively poorer survival, with PRETEXT IV showing the worst outcomes (5 year OS 25.6%, $p<0.001$). Surgical resection was significantly associated with improved survival ($p<0.001$). AFP >10,000 ng/mL predicted inferior EFS ($p=0.045$), though OS differences were not statistically significant. On multivariate Cox regression, three factors retained independent prognostic significance. PRETEXT stage was the strongest adverse determinant (HR 2.68, 95% CI 1.86–3.86, $p<0.001$), surgery was protective (HR 0.77, 95% CI 0.68–0.88, $p<0.001$), and age group independently predicted poorer outcomes, with younger patients showing a 63% higher hazard compared to those >10 years (HR 1.64, 95% CI 1.09–2.46, $p=0.018$). AFP and risk stratification did not retain statistical significance in the multivariate model.

Abstract 355

Phase I Dose-Escalation Trial of Irinotecan in the VIT Regimen Guided by UGT1A16 Genotype for Relapsed/Refractory Pediatric Solid Tumors

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Purpose: Vincristine, irinotecan, and temozolomide (VIT) is a standard salvage chemotherapy regimen for relapsed/refractory (R/R) pediatric solid tumors. UGT1A16 gene polymorphism determines irinotecan-related toxicity and efficacy in Chinese children. However, the genotype-specific irinotecan dose in the VIT regimen based on the UGT1A16 genotype is still undefined. This study aimed to determine the maximum tolerated dose (MTD) of irinotecan in Chinese pediatric solid tumor patients with the UGT1A16 G/G wild - type (WT) receiving VIT.

Methods: Pediatric patients (0-18 years) with R/R solid tumors and measurable/evaluable lesions were enrolled after UGT1A16 genotyping, with only UGT1A16 G/G WT patients eligible. VIT regimen was administered every 21 days: vincristine 1.5 mg/m² (max 2 mg, intravenously (IV), D1), temozolomide 100 mg/m²/d (IV, D1–5), and irinotecan (IV, D1–5) with dose escalation per a standard 3+3 cohort design (dose levels: 50, 65, 80, 95, 110 mg/m²/d). The primary endpoints were MTD and dose-limiting toxicity (DLT); secondary endpoints included treatment-related adverse events (TRAEs) and objective response rate (ORR).

Results: A total of 46 patients were genotyped, of whom 22 (47.8%) had the UGT1A16 G/G WT genotype, and 15 were finally enrolled. Two patients exhibited DLTs at the 80 mg/m²/d irinotecan level: one had grade 3 abdominal pain, another one had grade 3 diarrhea. The MTD of irinotecan was 65 mg/m2/d. All TRAEs were manageable: 13 (86.7%) patients experienced grade 1–2 TRAEs, with anorexia (80.0%, 12/15), diarrhea (73.3%, 11/15), and nausea (66.7%, 10/15) as the most common. No treatment-related deaths occurred. Among 9 patients with measurable/evaluable lesions, the ORR was 55.5%, including 1 complete response (CR, 11.1%), 4 partial responses (PR, 44.4%), and 3 stable diseases (SD, 33.3%).

Conclusion: Chinese children with UGT1A16 G/G WT when receiving the VIT salvage regimen, the MTD of irinotecan is 65 mg/m²/d. This study providing a clinically evidence-based irinotecan dose recommendation for this specific pediatric population.

Abstract 356

Prospective Study on Efficacy of Modified Treatment Regimens for Hepatoblastoma in Chinese Children

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Purpose: Hepatoblastoma (HB) is the most common primary malignant liver tumor in children, and risk-stratified treatment strategies are crucial for improving patient outcomes. This prospective study aimed to analyze the efficacy of modified treatment regimens for hepatoblastoma in Chinese children.

Methods: A total of 90 eligible hepatoblastoma patients admitted to Sun Yat-sen University Cancer Center from January 2018 to December 2025 were enrolled in this prospective study. Risk stratification was performed according to the Children's Hepatic Tumor International Collaboration (CHIC) classification. Patients with low risk received 4 courses of chemotherapy with single-agent cisplatin or C5V regimen (cisplatin, 5-fluorouracil, and vincristine). Intermediate-risk patients were treated with 6 courses of either SIOPEL-3 HR or C5VD regimen(cisplatin, 5-fluorouracil, vincristine and Pirarubicin). For high-risk patients, modified SIOPEL-4 regimen was adopted, with or without sorafenib combination therapy.

Results: Among the 90 analyzable cases, 17 were low-risk, 35 were intermediate-risk, and 38 were high-risk. In the low-risk group, only 1 case had recurrence, with an overall survival (OS) rate of 100%. For intermediate-risk patients, the objective response rate (ORR) of SIOPEL-3 HR regimen was 100%, while that of C5VD regimen was only 72.4%. The 3-year event-free survival (EFS) and 3-year OS of the intermediate-risk group were 86.4% and 96.2%, respectively. In the high-risk group, the ORR was 68.4%, with a 3-year EFS of 44.3% and a 3-year OS of 75.4%.

Conclusion: The modified risk-stratified treatment regimens based on CHIC classification show favorable efficacy in Chinese children with hepatoblastoma. Low-risk patients achieve excellent survival outcomes, intermediate-risk patients benefit more from SIOPEL-3 HR regimen than C5VD regimen, and the modified SIOPEL-4 regimen can improve the prognosis of high-risk patients to a certain extent. These findings provide a valuable reference for the clinical treatment of hepatoblastoma in Chinese children.

Abstract 357

LIVER TRANSPLANTATION FOR HEPATOBLASTOMA: ONE CENTER EXPERIENCE

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Purpose: Most cases of hepatoblastoma (HB) often represented as an advanced stage at diagnosing. Surgery, including liver transplantation, is the cornerstone of the comprehensive treatment of these patients. We present our experience of living-donor liver transplantation (LDLT) in HB.

Methods: Between 2008 and 2025, 162 children with HB underwent surgery in the Liver Transplant Department of the Petrovsky National Research Centre of Surgery (NRSC). Most patients required extensive surgery: trisectorectomies (n = 78, 48%) and LDLT (n = 42, 26%). Five patients underwent salvage liver transplantation (SLT). The median age at transplant was 37 months (range, 4–171). In one patient (3%) with HB diagnosed in underlying progressive familial intrahepatic cholestasis (PFIC) type IV. Two patients underwent AB0-incompatible LDLT. Synchronous pulmonary metastases (SLM) were detected in 18 (43%) patients of the transplant group. Three patients underwent inferior vena cava (IVC) prosthetics due to tumor thrombosis. All patients, except one with PFIC IV, received neoadjuvant chemotherapy (SIOPEL-3: n=11; SIOPEL-4: n=30). Ten patients underwent metastasectomy before LDLT. Basiliximab and methylprednisolone were used for induction immunosuppression in all recepients. The maintenance immunosupression involves a double-drug therapy with tacrolimus and corticosteroids. Steroids were discontinued in all patients within 1 to 3 months after transplantation.

Results: Five-year overall and relapse-free survival rates were: 86% and 84% for all patients with HB; 90% and 89% for the resection; and 74% and 72% in the LDLT group, respectively. Overall five-survival in recipients without/with SLM was 78%/68%. All patients who underwent SLT and required IVC prosthetics are alive.

Conclusion: Radical tumor removal is basis of a multidisciplinary treatment of HB. LDLT shows excellent results in patients with advanced HB, even in the presence of initial SLM, macrovascular invasion, and recurrent of disease.

Abstract 358

Late Effects in Survivors of Low-Risk Neuroblastoma

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Purpose: Patients with low-risk neuroblastoma (NB) have excellent long-term survival and are treated with low intensity therapy. However, even in this group, late adverse effects may occur and affect quality of life, highlighting the need for long-term follow-up. Aim to evaluate late complications and identify risk factors for long-term toxicity in survivors of low-risk NB

Methods: A retrospective cohort study included 399 patients with low-risk NB diagnosed between January 2012 and December 2019 and treated according to a modified German NB-2004 protocol at Dmitry Rogachev National Medical Research Center of Pediatric Hematology, Oncology and Immunology. Median follow-up was 80 months (range: 47–143). Late effects were analyzed using descriptive statistics and univariate and multivariate analyses

Results: Median age at diagnosis was 8 months(range:0.06–205), and median age at last follow-up was 101 months(range:38.5–305). Surgery was performed in 98% of patients, chemotherapy in 29%(median 2,range:1–4), and radiotherapy in 1.3%. Late complications included musculoskeletal disorders, n=100/378(26%): dental abnormalities n=64/362, spinal deformities n=29/378, etc; gastrointestinal disorders n=60/379(16%):constipation n=31/60, biliary tract disorders n=16/60, hepatic fibrosis n=7/60, etc; urinary system pathology, n=43/379(11%):neurogenic bladder n=13/43, recurrent urinary tract infections n=6/43, renal cysts n=5/43, solitary kidney after nephrectomy n=5/43, etc; neurological, n=27/380(7%):sensory impairment n=10/27, paresis n=15/27, plegia n=5/27, pelvic organ dysfunction n=11/27, etc; respiratory disorders n=28/379(7%):exertional dyspnea n=14/28, recurrent bronchitis n=15/28, recurrent pneumonia n=4/28; Horner syndrome n=23/380(6%), endocrine disorders n=15/383(4%):thyroid pathology n=14/15, adrenal insufficiencyn=1/15; cardiovascular pathology n=13/379(3%):arterial hypertension n=3/13, arrhythmia n=8/13, valvular heart disease n=3/13, etc and secondary malignancy n=1/399(0.25%) (atypical choroid plexus papilloma). Chemotherapy was associated with dental abnormalities(OR 2.3 (95% CI 1.3-4.1; p=0.004) and gastrointestinal pathology(OR 2.3 (95%CI 1.3-4.0;p=0.005). Spinal deformities were more frequent in patients with intraspinal tumor extension and after laminotomy(p<0.001) or thoracotomy(OR 11.2 (95%CI 4.6-27.3;p<0.001).

Conclusion: Late effects are frequent even in survivors of low-risk NB. Long-term follow-up and risk-adapted screening remain essential

Abstract 359

Minimally invasive and open surgery in abdominal neuroblastoma IDRF +

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Purpose: Accumulating evidence suggests that minimally invasive surgery (MIS) is feasible even in carefully selected IDRF+ NB. The purpose of this study is an analysis of perioperative and oncological outcomes after laparoscopic and open resection of abdominal NB IDRF+.

Methods: Perioperative, postoperative data and long-term outcomes of patients with abdominal NB IDRF + who underwent MIS (Group 1 = 46) and open surgery (Group 2 = 134) 2018 - 2023 were retrospectively analyzed. Propensity score matching (PSM, 1:1) was conducted to balance baseline characteristics in groups.

Results: After PSM the tumor volume (19 mL vs. 33 mL; p > 0.9), midline extension (17% vs. 28%; p>0,9), IDRF number (1 vs. 1), complications (6% vs. 12%; p = 0.9), gross total resection rates (96% vs. 97%; p = 0.9) and 3-year LRFS (82% vs. 81%); EFS (72% vs. 79%) and OS (88% vs. 87%) didn't differ.

Conclusion: MIS achieves comparable complication and gross total resection rates with open surgery in patients with small NB and 1-2 IDRFs. The tumor size/patient height ratio, extension across the midline, central tumor location, prior open surgery and chemotherapy should be investigated as surgical complexity factors.

Abstract 360

Laparoscopic surgery for pediatric malignant ovarian germ cell tumors after neoadjuvant chemotherapy: A single-center retrospective study.

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Purpose: To evaluate the clinical efficacy and oncological safety of neoadjuvant chemotherapy (NACT) combined with laparoscopic fertility-sparing surgery in pediatric patients with large malignant ovarian germ cell tumors (MOGCTs).

Methods: A retrospective analysis was conducted on seven female pediatric patients (median age: 8.8 years; range: 10 months to 13.4 years) treated between 2015 and 2023. All patients received 2-3 cycles of JEB as NACT. Following NACT, patients underwent laparoscopic (n=7) unilateral salpingo-oophorectomy.

Results: NACT achieved a median tumor volume reduction of 91% . All procedures were successfully completed without conversion to open surgery. Median operative time was 85 min with minimal blood loss. With a median follow-up of 54 months , all patients achieved 100% disease-free survival.

Conclusion: NACT effectively downsizes giant MOGCTs, facilitating safe laparoscopic resection, preserving fertility, and ensuring rapid recovery for subsequent therapy.

Abstract 361

Analysis of prognostic factors of hepatoblastoma in children

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Purpose: Summarize and analyze the clinical characteristics of pediatric hepatoblastoma and the related factors influencing prognosis.

Methods: A retrospective analysis was conducted on the clinical data of 388 children with HB who visited the hospital from May 2005 to May 2024, aiming to investigate the independent risk factors influencing the prognosis of HB.

Results: 1. The median follow-up time was 56 months. The 5-year OS was 71.9% and EFS was 62.6%. 2. AFP <100ng/ml or >1000ng/ml, platelet >400×10^9/L, PRETEXT stage IV, vascular invasion, distant metastasis, and multiple lesions in the liver were independent risk factors affecting prognosis.

Conclusion: The prognosis of hepatoblastoma is related to the AFP level, the platelet count at the initial diagnosis, the PRETEXT staging, as well as the presence of vascular invasion, distant metastasis, and multiple lesions within the liver. Under different risk factors, the survival and prognosis of the disease will vary.

Abstract 362

Endocrine and neurosensory late effects in children cured of nasopharyngeal carcinoma: low incidence of complications in a cohort of 40 patients

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Purpose: Nasopharyngeal carcinoma (NPC) is a rare epithelial malignancy, accounting for 1-3% of childhood cancers. The use of multimodal treatment protocols, including polychemotherapy, radiotherapy, immunotherapy, and surgery, achieves overall survival rates of approximately 70-90%. However, improved prognosis naturally brings the issue of minimizing late effects of anticancer therapy into sharp focus. Radiotherapy to the nasopharyngeal area inevitably involves the hypothalamic-pituitary region, potentially leading to hypothyroidism, growth hormone deficiency, and hypogonadism. Chemotherapeutic agents used in standard regimens (vinblastine, cyclophosphamide, platinum derivatives) also possess potential endocrine and neurosensory toxicity. Objective: To evaluate the frequency and pattern of late endocrine and neurosensory sequelae in children and adolescents cured of nasopharyngeal carcinoma according to clinical guidelines incorporating various chemotherapy regimens.

Methods: A retrospective single-center study was conducted at the Pediatric Oncology (Head and Neck Tumors) Department of the Research Institute of Pediatric Oncology and Hematology from 2020 to 2025. The study included 40 patients with newly diagnosed nasopharyngeal carcinoma. The median age at diagnosis was 14.4 years (range: 7-18 years). Histological examination revealed type III in 34 patients (85%) and type II in 6 patients (15%) according to the WHO 2005 classification of NPC. All patients received combined modality treatment including polychemotherapy, radiotherapy, and surgery. Chemotherapy regimens differed based on histological type: patients with undifferentiated nasopharyngeal carcinoma received a regimen including vinblastine, cyclophosphamide, bleomycin, and doxorubicin. Patients with type II morphology received a regimen of docetaxel and cisplatin. All patients underwent intensity-modulated radiotherapy (IMRT) to the primary tumor site and regional lymphatic drainage areas. Total radiation doses ranged from 50.4 Gy to 60 Gy depending on risk group. To assess late effects, all patients in remission underwent comprehensive evaluation. Endocrine assessment included evaluation of physical development (anthropometry with calculation of height SDS and body mass index), measurement of thyroid-stimulating hormone (TSH), free thyroxine (FT4), insulin-like growth factor-1 (IGF-1), and sex hormones (luteinizing hormone, follicle-stimulating hormone, testosterone, estradiol) according to age and sex; pubertal development was assessed using the Tanner scale. Neurosensory evaluation included pure-tone audiometry with hearing loss graded according to CTCAE version 5.0 criteria, assessment of renal function (glomerular filtration rate calculated by the Schwartz formula, serum creatinine and electrolyte levels)

Results: Comprehensive evaluation of 40 patients cured of nasopharyngeal carcinoma revealed no endocrine abnormalities in any case: growth parameters (mean height SDS -0.2), thyroid function (TSH, FT4), and pubertal development (Tanner stage) were within age-appropriate norms for all patients. Neurosensory complications were registered exclusively in the patient group (n=6) receiving cisplatin: grade 2 hearing loss (CTCAE) was observed in 2 patients (5%), and decreased glomerular filtration rate (<90 ml/min) in 3 patients (7.5%). In the patient group receiving vinblastine, cyclophosphamide, bleomycin, and doxorubicin (n=34), no oto- or nephrotoxicity was observed. No other late complications (visual impairment, trismus) were registered.

Conclusion: Analysis of late sequelae of NPC therapy demonstrates a clear dependence of the toxicity profile on the chemotherapy regimen. Cisplatin use is associated with the development of oto- and nephrotoxicity, while alternative regimens (vinblastine, cyclophosphamide, doxorubicin, bleomycin) are not accompanied by these complications. The use of modern radiotherapy techniques (IMRT/VMAT) allows minimization of the dose to the hypothalamic-pituitary region, which likely accounts for the absence of endocrine disorders in both patient groups.

Abstract 363

Surgical treatment of children with pancreatic tumors.

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Purpose: Background: Pancreatic tumors (PT) are rare neoplasms with low malignant potential, accounting for less than 2% of tumors. Objective: To determine the tactics and possibilities of surgical treatment of children with pancreatic tumors.

Methods: From 2007 to 2025, 81 patients diagnosed with pancreatic tumor underwent surgery. We analyzed clinical and diagnostic data, surgical procedures, treatment outcomes, and follow-up time. Results: Most patients (66) had solid pseudopapillary pancreatic tumors. Of these, 61 were girls, with a mean age of 13.8 years. The disease course was asymptomatic in most cases. According to examination data, the tumor was located in the tail of the pancreas in 25 patients,

in the body in 25, and in the head in 16. The maximum tumor size was 579 cm3.

Results: Other morphological forms of pancreatic tumors were identified in 15 patients: neuroendocrine tumor (8 patients), pancreatoblastoma (3 patients), paragangloma (1 patient), myeloid sarcoma (1 patient), acinar cell carcinoma (1 patient), and lymphangioma (1 patient). Twenty-one children underwent a Whipple procedure, 17 underwent distal pancreatectomy, 24 underwent central pancreatectomy, 4 underwent enucleopectomies, 12 underwent laparoscopic distal pancreatectomy, and 3 were diagnostic surgeries. Complications developed in 18 patients: type C fistula (3 patients), type B fistula (5 patients), and others. All patients are alive with no signs of disease recurrence.

Conclusion: Pancreatic tumors are a rare disease in children, typically occurring in girls during puberty. Surgery is the primary treatment option, although endoscopic surgery is possible. However, the indications for this type of treatment and the risk of postoperative complications must be carefully assessed.

Abstract 364

COMPUTED TOMOGRAPHY(CT) SIGNS CORRELATION OF BIOPSY IN PATIENTS WITH PEDIATRIC RETROPERITONEAL TUMORS

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Purpose: According to the 2022 estimates of the Global Cancer Observatory, 280,000 children (aged 0–19 years) are diagnosed with cancer worldwide, with more than 105,000(37.5%) dying from it. Asia. National Cancer Institute ranks Neuroblastoma 4th and renal tumors 6th among all childhood cancers. At National Center for Maternal and Child Health (NCMCH), 98 children registered retroperitoneal tumors and the steadily increasing annual incidence of these conditions highlights the critical need for this research. Objective: The study is aimed to compare CT signs and biopsy of pediatric retroperitoneal tumors

Methods: This retrospective study was conducted between 2020 to 2025 by sampling 99 children who were diagnosed with retroperitoneal tumor by undergoing contrast CT scan and confirmed biopsy at NCMCH and Gurvan gal hospital. The analysis was done selecting 31 cases according to the inclusion and exclusion criteria. Statistical analysis was performed Microsoft excel and BMI SPSS.

Results: Among 31 patients. The mean age was 2.92±3.57 years, 77.4%(n=24) were boy, 22.6%(n=7) were girl. Pediatric retroperitoneal tumors were diagnosed by contrast CT scan (Philips Ingenuity Core CT machine with 128 slices in NCMCH and Philips CT scan Brilliance 64 slices in Gurvan Gal Hospital). Neuroblastoma 54.8%(17±2.85), Willms 32.3%(10±2.85), others 12.9%(4±2.85). CT signs; components- cystic 9.7%(3±0.67), solid 25.8%(8±0.67), mixed 64.5%(20±0.67), margins- well defined 54.8%(17±0.5), ill defined 45.2%(14±0.50), septa- 9.7%(3±0.3), clacification-45.2%(14±0.5), fat content-9.7%(3±0.3), necrotic lession- 29.0%(9±0.46) , contrast enhancement-homogeneous 6.5%(2±0.83), heterogeneous 83.9%(26±0.83), wall enhancement 3.2%(1±0.83), non enhancement 6.5%(2±0.83) . The association between suspected diagnosis on CT scan and biopsy report were statistically signification when assessed by Pearson Chi-Square test ($p<0.001$, $x2=54.402$), Cohen`s kappa was fair agreement($k=0.243$).

Conclusion: The result of this research, The strong correlation between CT signs and biopsy of pediatric retroperitoneal tumors($p<0.001$). In conclusion, CT imaging proves to be a highly effective diagnostic tool for pediatric retroperitoneal tumors, especially for Neuroblastoma and Willms.

Abstract 365

Analysis of bone health in pediatric and adult patients with TRK fusion solid tumors treated with larotrectinib

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Purpose: Tropomyosin receptor kinase (TRK) signaling plays a crucial role in bone homeostasis, regulating bone formation and remodeling through nerve growth factor-activated signalling.1–4 The effect of TRK inhibition on bone homeostasis is not fully understood; however, serious fractures with or without trauma or injury have been reported in patients who received TRK inhibitor treatment for TRK fusion cancer, particularly in pediatric patients.5–9 Larotrectinib is a first-in-class, highly selective, central nervous system (CNS)-active TRK inhibitor with marketing approval for tumor-agnostic use in pediatric and adult patients with TRK fusion cancer.10,11 While other TRK inhibitors also target the TRKA, TRKB, and TRKC receptors, their kinase selectivity profiles differ.6–11 Larotrectinib was designed to avoid the off-target inhibition of kinases, such as ALK, ROS1, JAK2, and FGFR1, observed with other marketed TRK inhibitors.6–11 This study evaluated the fracture incidence and bone health of pediatric and adult patients with locally advanced or metastatic TRK fusion cancer treated with larotrectinib.

Methods: Following protocol amendments in 2021 in the NAVIGATE (NCT02576431) and SCOUT (NCT02637687) clinical trials, bone health data were prospectively collected in newly enrolled patients in both studies and in ongoing patients in SCOUT who reconsented for inclusion in the bone health cohort. Both adult and pediatric patients with locally advanced or metastatic TRK fusion cancer were included. The bone health analysis population included all treated patients (in each trial) with ≥1 valid dual-energy X-ray absorptiometry (DXA) or serum bone marker assessment. Assessments included: Bone mineral density (BMD) or content (BMC) by DXA (Z-scores in children and young adults, and T-scores in adults). Bone maturation by X-ray (SCOUT only). Bone turnover and bone mineral metabolism serum markers. Fracture incidence and severity (graded by Common Terminology Criteria for Adverse Events v4.03). Height (NAVIGATE) and growth curves (SCOUT).

Results: Participants: Out of 369 patients in the SCOUT (0–21 years) and NAVIGATE (≥18 years) studies, 67 patients (35 pediatric, 32 adult) were included in the bone health cohort. Primary tumor types: Most common were CNS tumors (25%) and soft tissue sarcoma (22%). Baseline bone conditions: 31% had prior conditions affecting bone health (e.g., fractures, osteoporosis/osteopenia, vitamin D deficiency, arthritis, etc.). Fractures: Occurred in 8% of the overall cohort and 9% of the bone health cohort. All fractures occurred in pediatric patients and were mainly trauma-related (falls/sports). All were mild (Grade ≤2), with no treatment discontinuation or dose modification required. Bone mineral density (BMD): No significant decline in BMD, Z-scores, or T-scores in either adults or children. No progression to osteoporosis observed. Bone metabolism markers: Minor fluctuations observed, but no clear adverse trends. Most values remained within normal ranges. Growth and development: No significant height loss in adults. Children showed normal growth trajectories. Bone age: Pediatric bone age was generally consistent with chronological age.

Conclusion: In this subset of patients assessed for bone health parameters from 2 clinical trials, larotrectinib did not adversely affect bone health in adult or pediatric patients with TRK fusion cancers. There was no increase in the incidence of fractures relative to that expected for patients with cancer.12–15 No detrimental effects on BMD Z- or T-scores were observed over time. Larotrectinib did not appear to have a negative impact on biochemical markers of bone remodeling or bone health in children or adults, nor on growth or development in pediatric patients. These results reinforce the favorable risk–benefit profile of larotrectinib as a standard-of-care therapy for patients with TRK fusion cancers.

08. IMPLEMENTATION SCIENCE — ORAL PRESENTATION

Abstract 366

GERM LINE PREDISPOSITION IN PEDIATRIC ONCOLOGY PATIENT: A REVIEW FROM SAUDI ARABIA

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Purpose: Cancer predisposition syndromes (CPS) contribute substantially to pediatric oncogenesis. In populations with high consanguinity rates, such as in Saudi Arabia, the prevalence and spectrum of CPS may differ from those reported

in Western cohorts. This study aimed to describe the frequency and characteristics of germline variants in a pediatric oncology population.

Methods: We conducted a retrospective cohort study of 103 pediatric patients with cancer who were referred for germline genetic testing over a 5-year period at King Fahad Specialist Hospital, Dammam, Saudi Arabia. Germline testing utilized targeted gene panels, whole-exome sequencing, and whole-genome sequencing, with results interpreted using contemporary international standards for pediatric oncology germline analysis.

Results: The median age at cancer diagnosis was 5 years. Of 102 patients who completed germline testing, 53 children (51%) were confirmed to have CPS. Among these, 23 patients (45%) habored Pathogenic or likely pathogenic variants , and 30 patients (55%) had variants of unknown significance. Recurrently affected genes included TP53 (15%), ATM (12%), MSH6 (9%). And BRCA2 (6%). A family hisory of cancer was reported in 68 patients (66%), while consanguinity was documented in 40 families (39%). Notably, testing prompted by second primary cancers (predominantly brain tumors and AML) identified CPS in 6 patients (6%), primarily due to mismatch repair deficiency syndrome. Genetic testing was primarily prompted by specific tumor histology rather than family history

Conclusion: Germline pathogenic variants were identified in a substantial proportion of pediatric patients with cancer in this Saudi cohort, suggesting a distinctive genetic architecture influenced by consanguinity and potential founder effects. The considerable burden of variants of uncertain significance underscores challenges of variant interpretation in underrepresented populations. Incorporation of comprehensive germline testing into pediatric oncology care may support therapy adaptation, family counselling, and development of population-specific surveillance strategies.

Abstract 367

Diagnostic Value of Pathogen Metagenomic Next-Generation Sequencing (mNGS) for Early Bloodstream Infections in Pediatric Allogeneic Hematopoietic Stem Cell Transplantation Recipients

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Purpose: Early bloodstream infection (EBSI) remains a principal determinant of morbidity and mortality after paediatric allogeneic haematopoietic stem-cell transplantation (allo-HSCT). Metagenomic next-generation sequencing (mNGS) offers a culture-independent, high-throughput approach for comprehensive pathogen identification. We investigated the diagnostic performance of plasma mNGS for EBSI in children undergoing allo-HSCT.

Methods: In this single-centre, retrospective cohort study, we consecutively enrolled 161 transplant episodes performed in 157 paediatric patients at the Pediatric Hematology and Oncology Center, Institute of Hematology, Chinese Academy of Medical Sciences, between January 2024 and April 2025. Blood cultures and plasma mNGS were systematically collected from conditioning initiation to day +30 post-transplant. Group comparisons employed Fisher's exact test.

Results: Blood-culture-confirmed BSI was documented in 18 of 161 episodes (11.2%). Disease-specific incidence rates were: ALL 19.4 % (6/31), AML 10.5 % (4/38), acquired aplastic anaemia/other marrow failure 7.4 % (4/54), myelodysplastic syndrome 4.5 % (1/22) and other haematologic malignancies 25.0 % (3/12). Donor-specific BSI rates were 11.5 % (7/61) after haploidentical, 15.3 % (11/72) after unrelated cord-blood and 0 % (0/34) after matched-sibling transplantation. There were no statistically significant differences were found across disease subtypes (P = 0.31) or donor sources (P = 0.60).Predominant blood-culture isolates were Streptococcus mitis, Klebsiella pneumoniae and Escherichia coli. Plasma mNGS was obtained in 39 episodes, yielding 22 positives (56.4 %). Among febrile episodes, blood culture was positive in 9/39 (23.1 %). mNGS identified bacterial infection in 8, viral infection in 6 and combined bacterial–viral infection in 8 cases. Leading bacteria were K. pneumoniae, Pseudomonas aeruginosa and S. mitis. Dominant viruses were HHV-6B, CMV and BK virus. Disease-stratified mNGS positivity was 100 % (5/5) for ALL, 75.0 % (6/8) for AML, 33.3 % (1/3) for MDS and 37.5 % (6/16) for severe aplastic anaemia. AML/ALL group exhibited significantly higher positivity than the combined MDS/SAA group (84.6 % vs 36.8 %, P = 0.017). Donor-stratified mNGS positivity was 52.1 % (12/23) after unrelated cord blood, 69.2 % (9/13) after haploidentical and 33.3 % (1/3) after matched-sibling transplantation (P > 0.05). Eight mNGS-positive patients with neurological symptoms underwent cerebrospinal fluid mNGS; seven were positive, revealing S. mitis with HHV-6 (two), HHV-6 alone (two), S. mitis alone (one), P. aeruginosa (one) and K. pneumoniae (one). Two infection-related deaths occurred before day +30.

Conclusion: mNGS demonstrates high diagnostic value for early infections post-HSCT, particularly in AML/ALL patients, with superior detection of mixed and viral infections compared to blood culture. CNS involvement was common in mNGS-positive cases, highlighting the need for comprehensive pathogen screening in high-risk pediatric HSCT recipients.

Abstract 368

Evaluating Palliative Care Knowledge, Attitudes, and Competencies in Medical Students: A Needs Assessment for Curriculum Development

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Purpose: Palliative care (PC) is an integral part of comprehensive healthcare. However, PC integration in undergraduate medical education remains limited worldwide. This gap is specifically evident in the MENA region, where only three countries including Lebanon, provide structured PC training in medical schools. Additionally, there is significant lack in existing regional literature assessing PC knowledge, attitudes, and competencies especially among Lebanese medical students.

Methods: A cross-sectional study was conducted at the Lebanese American University Gilbert and Rose-Mary Chagoury School of Medicine over an 8-month period in 2025. An online survey was shared with all 256 registered medical students, assessing 4 key areas: demographics, PC knowledge (14-item composite score), attitudes toward PC education, perceived educational needs, and barriers to curricular integration. To evaluate independent predictors of PC Knowledge, a binary logistic regression was used.

Results: Responses were collected from 240 students (94% response rate; 55% female; median age 23). Only 8% reported high PC knowledge and around 38% had received structured PC training. Approximately half (52.8%) showed good knowledge. In multivariable analysis, independent predictors of higher knowledge included personal or clinical experience with PC patients (aOR 2.12, p=0.032), very good to excellent self-rated academic performance (aOR 2.99, p=0.007), and specialty preference outside conventional tracks (aOR 2.27, p=0.009). Significant deficiencies in knowledge gaps were detected in symptom management (20% correct) and end-of-life communication (5% correct). The vast majority (93%) favored mandatory PC education, 94% preferred longitudinal curriculum inclusion, and 93% endorsed learning in clinical situations. Merely 8% were comfortable navigating end-of-life conversations with patients.

Conclusion: This study presents the first assessment of PC knowledge, attitudes, and educational needs among Lebanese medical students. Results revealed moderate knowledge with gaps in symptom management and terminal care communication, confirming that PC education is not yet adequate and providing a foundation to build a structured curriculum across MENA and Asia.

Abstract 369

Implementation of Pediatric Early Warning Systems in Pediatric Oncology unit in Pakistan: Impact on Clinical Deterioration events and Outcomes

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Purpose: The Pediatric Early Warning System (PEWS) is a structured bedside tool designed to identify early physiological deterioration and facilitate timely escalation of care. This study aimed to evaluate the impact of PEWS implementation on early detection of clinical deterioration, mortality associated

with clinical deterioration events (CDEs), and healthcare provider compliance in a pediatric oncology unit in Pakistan

Methods: We conducted a prospective interventional quality improvement study in the Pediatric Hematology/Oncology unit at The Children's Hospital & University of Child Health Sciences Lahore. Pediatric patients aged 1 month to 16 years admitted during the study period were included. A locally adapted PEWS tool was implemented alongside structured staff training using a train-the-trainer model. Ongoing mentorship was provided through collaboration with St. Jude Children's Research Hospital under Project PASHA, with funding from Foundation S. Clinical deterioration events (CDEs), defined as unplanned transfer to higher levels of care, need for intensive care interventions on the ward, or ward death, were monitored before and after implementation. Data were analyzed using descriptive statistics comparing pre-implementation, pilot, and post-implementation phases.

Results: During the pre-implementation phase, 147 of 936 admitted patients required unplanned transfer to a high dependency unit, with a mortality rate of 52%. Following PEWS implementation, earlier recognition of deterioration enabled timely intervention. Mortality associated with CDEs decreased to 13% during the pilot phase and 19% during the implementation phase. A total of 1,439 healthcare providers (1,251 nurses and 188 physicians) were trained. Documentation and scoring accuracy improved, with error rates reduced to 5% and 11% during the pilot and implementation phases, respectively.

Conclusion: PEWS implementation improved early detection of clinical deterioration, reduced mortality, and strengthened healthcare provider capacity in a resource-limited pediatric oncology setting, demonstrating the feasibility of scalable quality-improvement initiatives in LMIC pediatric cancer programs.

Abstract 370

A Prospective Screening and Evaluation of Cancer Predisposition Syndrome in Children with Malignancy

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Purpose: Cancer predisposition syndrome (CPS) are present in 8-10% of children with malignancy. Children diagnosed with CPS have wide-ranging requirements beyond that of children without CPS, including possible modification of chemotherapy, radiotherapy, and surgery; change in prognostication, and requirement of follow up. Family screening and counseling is required in some.

Methods: Prospective evaluation of 379 consecutive children diagnosed with cancer within last 2 years, either on therapy or completed therapy, was done over 1 year (April 2025-March 2026). Children positive for screening by Modified Jongmans criteria or MIPOGG app were counseled for need for whole exome sequencing (WES) for cancer predisposition syndrome. WES was done from peripheral blood from children in remission, for germline genetic mutation testing.

Results: Seventy-six children (20%) were positive for screening (requiring genetic evaluation) by either criterion, and were offered genetic testing. It was accepted by 71 patients, and refused by 5 patients. WES was done for 71 patients, and was positive in 25 (35%) for pathogenic mutations. The cancer diagnosis of tested patients was acute lymphoblastic leukemia (20%), other leukemias or lymphoma (5%), retinoblastoma (17%), hepatoblastoma (15%), Wilms tumor (13%), brain tumors (10%), with 20% being other solid tumors. The most common affected gene causing cancer predisposition syndrome was RB1 (33%), TP53, NF1, and BRCA2 in 14% each, followed by WT, DICER, APC, CBL, TS2, APC and TERT. Three patients had more than one pathogenic mutation. Adverse outcome was noted more frequently in children with CPS as compared to children without CPS, including relapse, progression or toxic deaths.

Conclusion: Acceptance rate for genetic evaluation was high in our population. A total 6.6% children with cancer were positive for cancer predisposition syndrome and need further detailed planning and follow up. They have been offered family testing and planned for subsequent screening. This incidence is lower than reported in the western literature.

Abstract 371

Bone Sarcomas in Armenia: Impact of Centralized Multidisciplinary Care and Telemedicine-Supported Multidisciplinary Review

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Purpose: Bone sarcomas are rare and aggressive malignancies that require multidisciplinary care, delivery of which in resource-limited settings remains challenging due to limited expertise and diagnostic capacity. Since 2019, pediatric cancer care in Armenia has been centralized, with regular multidisciplinary tumor board discussions supported by international telemedicine collaboration.

Methods: We analyzed a retrospective-prospective cohort of patients with musculoskeletal sarcomas diagnosed and/or treated in Armenia from 2008 to 2021, focusing on bone sarcomas. We compared diagnostic and treatment-related indicators before and after 2019.

Results: Among 200 patients with musculoskeletal sarcomas, Ewing sarcoma and osteosarcoma accounted for 31.5% and 23.5% of cases, respectively. Bone sarcomas were more common in patients younger than 18 years (60.6%). The mean diagnostic delay for bone sarcomas was 98 days. Following centralization, musculoskeletal MDT discussion increased from 16.0% to 81.4%, pathology review from 24.0% to 88.4% (p=0.0001), while treatment refusal decreased from 22.0% to 10.3% (p=0.0415). Pathology reassessment changed the diagnosis in 30% of reviewed cases. The overall 3-year survival for bone sarcomas was 52.4%. Among 36 patients with localized bone sarcomas who underwent surgical treatment, 22 underwent limb-sparing surgery and 14 underwent amputation. The 3-year overall survival was higher in the limb-sparing group compared with the amputation group (74.0% vs 41.7%; HR=5.02, p<0.003). This difference may reflect differences in tumor location and disease extent influencing surgical decision-making and eligibility for limb-preserving procedures.

Conclusion: Centralized care supported by international telemedicine collaboration improved key quality-of-care indicators for bone sarcomas in Armenia. This approach offers a feasible model for strengthening pediatric oncology systems across LMICs.

Abstract 372

Innovation in Pediatric Pain Management: Knowledge, Attitudes, and Perceptions of Lebanese Healthcare Professionals Regarding the Use of Virtual Reality for Pain and Anxiety Management in Children.

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Purpose: Pain and anxiety prior to and during procedures, are reported by children and families as distressing symptoms. Virtual Reality (VR) is emerging as an effective distraction tool by providing an innovative, immersive, psychology-based approach that shifts focus from pain through a three-dimensional simulated environment. Nevertheless, its use across Lebanese institutions has not been investigated. We aimed to explore attitudes of healthcare providers (HCPS) in Lebanon towards the use of VR in pain and anxiety management of children with cancer and hematological diseases prior to its implementation in a national pioneer project.

Methods: After obtaining IRB approval, this cross-sectional study used a bilingual survey validated by experts and distributed across several hospitals by Email and phone messages aimed to assess HCPs' knowledge, attitudes, and perceptions regarding VR for pain and anxiety management in children, prior to the structured education and implementation of VR in seven hospitals in a national pioneer project led by the CHANCE Association, a local NGO of public utility, active for 20 years.

Results: A total of 117 HCPs completed the survey, including physicians (41), nurses (47), trainees (12), and psychologists (5), predominantly females (81.8%). Participants held generally positive opinions regarding VR's therapeutic significance. Moderate familiarity (M=3.69/5) and self-confidence (M=3.51/5) were reported. VR's capacity to improve patient collaboration received the highest

rating (M=4.03/5), followed by its effectiveness as a distraction tool (M=3.98/5). Perceived clinical viability was favorable (M=3.83/5), and only 12.8% expressed safety concerns. Notably, 23.9% reported no familiarity with any standard pediatric pain assessment tool, emphasizing a broader educational deficit.

Conclusion: Our findings showed that Lebanese HCPs are open to using VR in pediatric pain management. The key challenge is not unwillingness or hesitancy, but lack of exposure and training, precisely the gap that the CHANCE Association's structured VR initiative is designed to bridge.

Abstract 373

From Framework to Function: Myanmar's National Implementation of the WHO CureAll Strategy for Childhood Cancer

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Purpose: Myanmar was among the first countries engaged as a focus country under the WHO Global Initiative for Childhood Cancer (GICC) in 2018. The country adopted CureAll as a structured framework to reform childhood cancer control, led by the Ministry of Health in collaboration with WHO, St. Jude Global and technical partners. Myanmar's national implementation of CureAll was analyzed across four pillars and three enablers as a health systems strengthening model.

Methods: CureAll project documentation, national policies, workforce records, and financing reforms were reviewed and mapped across the CureAll pillars: Centers of Excellence and Care Networks (C), Universal Health Coverage (U), Regimens for Management (R), and Evaluation and Monitoring (E), alongside enablers of Advocacy, Leveraged Financing, and Linked Policies and Governance.

Results: Under the C pillar, Myanmar expanded a coordinated childhood cancer network linking two tertiary centers with 41 satellite hospitals and implemented structured pediatric hematology workforce training for 115 providers and 18 nurses. Notably, satellite hospitals served up to 30% of newly diagnosed cases, providing critical system resilience and reducing family burden during natural calamities and political turmoil. Under the U pillar, a Costed National Plan and Minimal Essential Package for six index cancers were developed for public financial protection. Under the R pillar, the Curing Safely Resource Guide and Medication Information Sheets were translated to support context-appropriate and evidence-based management across 41 satellite hospitals. Under the E pillar, hospital-based and population-based cancer registry systems, including SJCARES Registry implementation and strengthened documentation at shared-care centers were established. By December 2025, approximately 4,550 children have been newly diagnosed and continue to benefit from the development and implementation of the CureAll framework.

Conclusion: Myanmar demonstrates how integrated, cross-pillar application of the CureAll framework can translate a global policy framework into coordinated national action. This model offers practical insights for countries operationalizing GICC toward 2030 targets.

Abstract 374

HYPERFERRITINEMIC SEPSIS IN PEDIATRIC ONCOLOGY: A STUDY OF CLINICAL CHARACTERISTICS, ORGAN DYSFUNCTION, AND OUTCOMES

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Purpose: Hyperferritinemic sepsis represents a distinct subset of sepsis characterized by excessive immune activation, marked hyperferritinemia, cytopenias, liver dysfunction, and coagulopathy. Children with cancer are particularly susceptible due to chemotherapy-induced neutropenia, recurrent infections, and immune dysregulation. However, data on this entity in pediatric oncology are limited.

Methods: We conducted a retrospective analysis of pediatric oncology patients with hyperferritinemic sepsis admitted to our centre. Clinical characteristics, laboratory parameters, infectious etiologies, organ dysfunction, treatment strategies, and outcomes were reviewed.

Results: Eighteen pediatric oncology patients with hyperferritinemia not fulfilling HLH/MAS criteria were included. Females comprised 55.6% (n=10). Acute leukaemia was the most common underlying malignancy, with B-cell acute lymphoblastic leukaemia accounting for 55.6% (n=10). Fever and anaemia were present in all patients, while pallor was observed in 88.9% (n=16). While bacterial bloodstream infections were identified in 28% of cases, endemic viral co-infections (including Dengue NS1+, COVID-19, and Influenza A) triggered 39% of episodes. Haematological abnormalities included neutropenia in 88.9% (n=16), severe neutropenia in 66.7%, and thrombocytopenia in 88.9% (n=16). Organ dysfunction was common, with liver dysfunction in 94.4% (n=17), renal dysfunction in 11.1% (n=2), and coagulopathy in 16.7% (n=3). Respiratory support was required in 33.3% (n=6), including mechanical ventilation in 16.7% (n=3), while vasopressors were required in 22.2% (n=4). Viral and fungal infections were identified in 16.7% (n=3) and 22.2% (n=4) of cases, respectively. The median peak ferritin level was 14,882 ng/mL (IQR 6,249–33,878). Immunomodulatory therapies, including intravenous immunoglobulin, corticosteroids, tocilizumab, and plasma exchange, were administered to 77.7% of patients (n=14). Overall mortality was 22.2% (n=4).

Conclusion: Hyperferritinemic sepsis represents a severe hyperinflammatory phenotype in pediatric oncology patients, particularly in children with acute leukemia and febrile neutropenia. Outcomes appear to correlate more strongly with the extent of organ dysfunction than with ferritin levels alone. Early recognition may facilitate timely immunomodulatory interventions and improve outcomes.

Abstract 375

Staged Evolution of Enhanced Recovery After Surgery Protocols in Pediatric Extralobar Pulmonary Sequestration: A 10-Year Single-Center Experience with 142 Cases

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Purpose: Extralobar pulmonary sequestration (EPS) is an ideal candidate for enhanced recovery after surgery (ERAS) due to its anatomical independence. However, long-term, staged experiences with ERAS implementation in pediatric EPS are lacking. This study aimed to evaluate the clinical outcomes of a stepwise, four-stage ERAS protocol evolution over a decade at a single center.

Methods: A retrospective analysis was conducted on 142 pediatric patients with pathologically confirmed EPS who underwent thoracoscopic surgery between January 2016 and March 2026 at Beijing Children's Hospital, Capital Medical University. Patients were divided into four sequential groups based on the progressive implementation of ERAS measures: Group A (traditional management, all tubes retained, n=21), Group B (thoracoabdominal drainage tubes removed, n=38), Group C (complete tube removal, i.e., no gastric tube, urinary catheter, or central line, n=69), and Group D (Tubeless, i.e., complete tube removal plus avoidance of endotracheal intubation using a laryngeal mask, n=14). Baseline characteristics, perioperative outcomes, opioid and rocuronium consumption, and complications were compared.

Results: The four groups were comparable in baseline demographics, clinical presentations, and lesion characteristics (all $P>0.05$). A significant stepwise reduction in postoperative hospital stay was observed: Group A, 7.0 (IQR 7.0–7.0) days; Group B, 3.0 (IQR 1.0–3.0) days; Group C, 2.0 (IQR 2.0–3.0) days; and Group D, 2.0 (IQR 2.0–3.0) days ($P<0.001$). Anesthesia duration, anesthesia preparation time, anesthesia recovery time, and operative time all progressively decreased, with Group D showing the shortest times ($P<0.001$ for all). Opioid consumption was nearly eliminated in Group D [0.1 (IQR 0.0–0.2) μg/kg], significantly lower than the other groups ($P<0.001$). Rocuronium use was zero in Group D, also significantly lower ($P<0.001$). White blood cell counts on postoperative day 1 were similar across groups ($P=0.734$). Complication rates did not differ significantly among the groups ($P>0.05$).

Conclusion: This 10-year staged experience demonstrates that the stepwise implementation of an ERAS protocol—from traditional tube retention to complete tube removal and finally to a Tubeless technique—is safe and feasible in pediatric

EPS. This progressive approach significantly accelerates postoperative recovery, shortens anesthesia and operative times, and reduces opioid and neuromuscular blocker usage without increasing complications. The Tubeless technique offers substantial advantages and represents a promising direction for future practice.

Abstract 376

TIMELY LEAP: Reducing Delays in Multidisciplinary Care for Pediatric Cancer Survivors in Thailand

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Purpose: Risk-based survivorship care requires a comprehensive and well-coordinated multidisciplinary team (MDT) follow-up. In resource-constrained settings, fragmented transitions from active treatment to survivorship care and understructure MDT coordination can lead to incomplete subspecialty evaluations and prolonged clinic visits. This study aimed to improve the completion of necessary MDT follow-up through structured and comprehensive MDT coordination while maintaining high satisfaction among survivors and healthcare providers (HCPs).

Methods: We conducted a prospective single-center quality improvement initiative, the TIMELY Late Effects Action Program (LEAP), using the Model for Improvement with iterative Plan–Do–Study–Act (PDSA) cycles. Interventions included establishing regular MDT meetings, structured tracking of three prioritized subspecialties within a standardized survivorship care bundle, centralized survivors' appointment, and clearly defined MDT roles. The primary outcome was timely completion of prioritized subspecialty follow-up. Mean clinic visit duration was evaluated as an efficiency measure. Survivors' and HCPs' satisfaction, measured using a 5-point Likert scale, served as balancing measures.

Results: Four MDT meetings were conducted, and two PDSA cycles were completed across two late-effects clinic visits involving 18 survivors. Baseline completion of prioritized subspecialty follow-up was 30%, increasing to 100% after two PDSA cycles. Mean clinic visit duration decreased from 242 minutes at baseline (n=21) to 202 minutes after cycle 1 (n=13) and 181 minutes after cycle 2 (n=5), representing a 25% reduction. HCPs' satisfaction score increased from 3.0 ± 1.41 in cycle 1 (n=3) to 5.0 ± 0.0 in cycle 2 (n=6). Survivors' satisfaction scores were 4.89 ± 0.32 in both cycles (n=18), with overall MDT meeting satisfaction of 4.76 ± 0.44 (n=15).

Conclusion: Timely LEAP improved completion of prioritized MDT follow-up and reduced clinic visit duration while maintaining high satisfaction among HCPs and survivors. Structured MDT coordination demonstrated measurable system-level improvements and offers a practical model for enhancing survivorship care delivery in resource-limited settings.

Abstract 377

Impact of Centralized Pediatric Pathology and Multidisciplinary Collaboration on Diagnostic Efficiency and Treatment Initiation: A Quality Improvement Study in Indonesia

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Purpose: Pediatric solid tumors are biologically aggressive and require rapid, precise diagnosis. At our institution, these cases were historically managed by general pathologists, potentially delaying the diagnosis. This study evaluates the impact of establishing a dedicated, centralized pediatric pathologist and a Multidisciplinary Team (MDT) at Hasan Sadikin General Hospital starting in 2023

Methods: We conducted a retrospective quality improvement analysis comparing decentralized (2023), transitional (2024), and centralized (2025) diagnostic phases. Key metrics included the number of solid tumor cases diagnosed (H&E and Immunohistochemistry), pathology Turnaround Time (TAT), and the rate of patients receiving definitive therapy initiation according to clinical registries in The Pediatric Hemato-Oncology Division

Results: In 2023, 80 pediatric oncology cases were diagnosed with an average TAT of 19 days. Following the appointment of a dedicated pediatric pathologist, the volume significantly increased to 151 cases in 2025, while the TAT was reduced to 14 days (a 26% improvement in efficiency). Clinical registry data mirrored this improvement: In 2023, only 30% (26/86) of diagnosed patients initiated definitive therapy. Following the full implementation of centralized pathology and MDT sessions, this rate improved significantly to 68% (111/162) in 2024 and reached 77% (115/148) in 2025

Conclusion: Centralizing Pediatric pathology services, combined with active MDT involvement, significantly improved diagnostic volume, efficiency and accelerates the initiation of definitive treatment. Despite lower case volumes compared to adults, the primitive and aggressive nature of Pediatric tumours necessitates specialized diagnostic focus. This model proves that subspecialisation in pathology is a vital component in improving the prognosis and survival rates of Pediatric Oncology patients in tertiary referral centres

Abstract 378

Quantifying intervals in childhood cancer diagnostic and care pathway for equitable cancer outcomes in Nepal: results of a prospective cohort study

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Purpose: We aimed to quantify the intervals across the diagnosis pathway and assess the determinants of delays in patients diagnosed with childhood cancer in Nepal.

Methods: DElays in CANcer care in Nepal (DECAN) study recruited a cohort of 168 consecutive childhood cancer patients newly registered over 6 months (June – November 2022) at the largest comprehensive oncology centre (B.P. Koirala Memorial Cancer Hospital) in Nepal.

Results: Overall, 79% (133/168) waited >30 days to diagnosis and 70% waited >60 days to treatment. The median total interval from symptom onset to treatment was 91 days (IQR 52–152).

Conclusion: We highlighted unacceptably longer delays from identification of symptom to accessing diagnostic and treatment services for any subtype of childhood cancer in Nepal. These data provide clear evidence that urgent mitigation measures should be a priority to improve early diagnosis and timely and quality treatment for childhood cancer patients with actions targeted at all levels of care (i.e., at individual level, at care providers level, at health system level). The study results can be utilized to inform professional and public health strategies and health policy to accelerate diagnosis, and in doing so, improve treatment outcomes as set in CureAll initiative and progress to meet the targets (10 out of 17) of sustainable development goals.

Abstract 379

Microbiological Profile, Resistance Patterns, and Outcomes of Culture-Positive Febrile Neutropenia in a Pediatric Oncology Unit

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Purpose: Febrile neutropenia (FN) is a major cause of morbidity and mortality in pediatric oncology. Unit-specific microbiological surveillance is essential to guide empirical therapy, antimicrobial stewardship, and infection control practices.

Methods: A review of culture data of patients with FN between January and December 2025 was conducted. FN episodes with positive cultures were analyzed for source, organism profile, resistance phenotype, central line-associated infections, and clinical outcomes.

Results: During the study period, 860 FN episodes occurred. Seventy-nine FN episodes (9%) were culture positive, yielding 110 isolates; 12 episodes were polymicrobial. Blood cultures accounted for 92 of the 110 positive samples. Gram-negative organisms predominated (69/110, 63%), gram-positive organisms (37/110, 34%), and fungi (4/110, 3.6%). Escherichia coli (21/69, 30%) and Klebsiella pneumoniae (13/69, 19%) were the commonest gram-negative isolates, while Staphylococcus aureus (17/37, 46%) and coagulase-negative staphylococci (11/37, 30%) predominated among gram-positive isolates. Multidrug resistance

was seen in 54/110 isolates (49%), extensive drug resistance in 15/110 (14%), and pan-drug resistance in 5/110 (4.5%). Among gram-negative isolates, ESBL-producing Enterobacterales were seen in 26%, carbapenem-resistant Enterobacterales in 34%, carbapenem-resistant Acinetobacter baumannii in 72%, and difficult-to-treat resistant Pseudomonas aeruginosa in 55%. Colistin retained 100% sensitivity among gram-negative isolates, whereas ciprofloxacin resistance was high at 78%. Methicillin resistance was seen in 75% of staphylococcal isolates, and 80% of Enterococcus isolates were vancomycin resistant. Catheter related bloodstream infection and central line associated bloodstream infection accounted for 10 episodes. Mortality was 18%. Five (36%) had gram-negative sepsis, four (28%) had gram-positive sepsis, and five had polymicrobial sepsis; 12/18 (86%) were resistant organisms.

Conclusion: Culture-positive FN was driven predominantly by gram-negative pathogens with a high burden of MDR/XDR phenotypes. This underscores the need for better gram-negative coverage in empirical therapy, robust infection control, central line care, antibiotic stewardship, and regular surveillance of local culture trends to guide antibiotic protocols.

Abstract 380

Recovery Timelines in Pediatric Oncology Patients with Acute Malnutrition

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Purpose: Low and middle income countries (LMICs) are faced with the battle of undernutrition of up to 45% and need to overcome this challenge to avoid undue morbidity and mortality in children on treatment for cancer. This study evaluates the recovery of nutritional status with a stepwise algorithm for nutritional rehabilitation in undernourished patients.

Methods: Fifty patients (7 years [0.3-15 years]; 74% males) who had undernutrition at diagnosis of a malignancy were analysed. Patients nutritional status was graded 1 to 4 based on WHO/IIPAN guidelines using Weight-for-Height, BMI, and MUAC. Intervention was (i) oral (OS) and (ii) enteral supplementation (EN). EN was employed in severely wasted patients, those meeting < 60% requirements and weight loss on OS. This analysis did not include patients who received parenteral nutrition. Recovery was defined as the transition from a malnourished state (Grades 2–4) to normal status (Grade 1).

Results: Grade of undernutriton : Grade(G) 2 : 6/50 ; G3 :26/50 (52%) ; G4 : 18/50(36%).Most patients received OS with thirteen receiving EN (G2 :1; G3: 4 & G4 : 8). Following structured intervention, 62% of the cohort showed a quantifiable improvement in their nutritional grade, with 23 patients (46%) becoming ‘normal’ over a 6 month observational period, median time being 115 days (IQR : 53-172).G4 showed a steady improvement : 7 improved to G3; 5 to G2; 4 to G1 with 2 remaining G4. Refusal for EN was high.

Conclusion: Targeted nutritional support can reverse acute malnutrition in pediatric oncology, with nearly half of patients achieving normal status within ~4.5 months. However, recovery requires sustained intervention, and early screening with proactive use of oral and enteral support is essential to optimize outcomes. Rigid refusal of nasogastric (NG) supplementation by caregivers or patients remains a significant barrier to adequate nutritional rehabilitation in pediatric cancer care.

Abstract 381

Applying Health Policy and Systems Science for Global Impact

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Purpose: Health policy and systems science offer salient opportunities to transform how we see, scale, and sustain strategies to address why the science of care and cure has not reached most children with cancer.

Methods: Anchored by locally relevant analogies, three applications of health policy and systems science are highlighted: shaping our vision, goal-setting with global agencies, and enabling champions across the SIOP Asia and global community. Across these three respective dimensions, we highlight the application of the Country Collaboration for Childhood Cancer Control (C5) tool, the CureAll framework underpinning the WHO Global Initiative for Childhood Cancer (GICC), and the National Cancer Control Planning integrating Children, Adolescents and Young Adults (NCCP iCAYA) program.

Results: Across the health policy and systems tools applied with the St. Jude Global Alliance community, 143 countries have engaged in collaborative systems and policy analysis, development, and strengthening. C5 supported over 140 prioritized national projects and informed national strategies, including Sri Lanka's first national strategy for children and adolescents with cancer. Over 500 individuals across 55 countries have engaged in participatory reporting of GICC implementation. NCCP iCAYA's four cohorts engaged over 550 multistakeholder champions and policymakers as part of 102 ministry-led teams from 53 countries.

Conclusion: Applying participatory health policy and systems science through resources such as C5, CureAll framework and NCCP iCAYA enables the global community to translate the science of care and cure for children and adolescents with cancer, providing a scalable model for strengthening health systems and aligning national and global efforts.

Abstract 382

A Collaborative Systems and Policy Workshop: Changing Systems, Saving Lives - The Journey Toward >60% Survival

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Purpose: The WHO Global Initiative for Childhood Cancer (GICC), launched in 2018 with St. Jude Children's Research Hospital and global partners, was established to address profound global inequities in childhood cancer outcomes by targeting at least 60% survival while reducing suffering.

Methods: Country and regional leads from three WHO regions represented at SIOP Asia shared insights, progress and impact from implemented participatory systems science approaches. The session incorporated interactive "How Might We" reflections and a Visioning Tree activity, leveraging appreciative inquiry and most significant change lenses.

Results: Country-led experiences demonstrate the application of participatory systems science tools to strengthen policy, systems, and data-driven decision-making across regions. NexPoS has connected over 1,200 collaborators across 115 countries for policies and systems science for better health.

Conclusion: Participatory systems science tools and systems thinking provide opportunities to build new linkages and narrow gaps in people-centered policy action. Empowered stakeholders engaged in inclusive priority-setting can enable science, compassion, hope, and togetherness for change for childhood cancer globally.

09. IMPLEMENTATION SCIENCE — POSTER

Abstract 383

MAINTENANCE OF SPECIFIC THERAPY CONTINUITY IN ADOLESCENTS AND YOUNG ADULTS WITH ONCOHEMATOLOGICAL DISEASES: FULL-CYCLE CLINIC MODEL

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Purpose: Second peak in malignant neoplasms (MN) incidence occurs in adolescents (13-19 y.o.). Physicians face a challenge of choosing between «pediatric» and «adult» treatment strategies for this age group. Through a

multidisciplinary approach N.N. Blokhin National Medical Research Center of Oncology, operating as a full-cycle clinic, provides personalized and continuous medical care for adolescents and young adults (AYA), including those reaching adulthood during treatment, with relapse prior to age 21 and patients aged 18-21 presenting with de novo tumors. To study the principle of «continuity» in specific therapy and its effectiveness in providing specialized medical care to AYA based on Research Institute of Pediatric Oncology and Hematology experience.

Methods: Retrospective cross-sectional analysis of 120 patients (aged 18+) treated inpatient (2010-2025). Particular focus on cases managed by a multidisciplinary team including adult surgical oncologists. Statistical methods (mean values and frequencies) and clinical data grouping were applied.

Results: 379 hospitalizations of 120 patients aged 18+ were analyzed (2010-2025), with a peak in 2023 (110 cases). Patients' age: 18,0 - 22,5 years, 85,7% were 18,0-18,9 y.o. The sample demonstrates leading pathologies: bone and soft tissue sarcomas (C40, C41, C49) – 42 patients (35%), lymphomas (C81, C83, C84) – 16 patients (13%) and acute lymphoblastic leukemia (C91.0) – 12 patients (10%). Rare cases: hepatoblastoma (C22.2), tumors of pleura (C38.2), cervix uteri (C53.9), and colon (C18.8). One female patient had two neoplasms: C73.9 at 18 years and C 49.2 at 19,3 years., both treated according pediatric protocols. Analysis of surgical activity revealed: 2019 - 794 operations (102 with adult surgeons), 2025 – 1159 (145 joint/adult teams). Solid tumors represented 98,9% of cases.

Conclusion: The full-cycle clinic model enables comprehensive methodological and technical medical support for AYA with MN, continuity of care and minimization of challenges associated with patients' transfer to adult services, improved the specific therapy effect and pediatric oncology care overall thanks to its human and material resources and multidisciplinary oversight (pediatric and adult specialists)

Abstract 384

Pediatric malignant spine tumours: the experience of National Medical Research Center Of Pediatric Oncology

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Purpose: Currently, no algorithms have been developed for the surgical treatment of malignant spinal tumors in children.We would like to share our experience and the results of surgical interventions.

Methods: The results of complex treatment of children under the age of 18 with malignant tumors of the spine during the last 12 years

Results: The report will highlight 12 years of experience in monitoring children with malignant tumors of the spine, which are observed in our clinic from all over the Russia. Strategy of emergency and planned decompression and stabilization surgery with rare clinical cases.

Conclusion: According to our results now we use induvidual approach in each case and futher multicentral research should be organised. The decision on treatment tactics should be made by a multidisciplinary team of oncologists, radiologists, neurosurgeons, and radiation therapists.

Abstract 385

Clinico-epidemiological patterns and survival outcomes of infant cancers in a low-resource setting: A 5-year retrospective cohort from Nepal

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Purpose: Infant cancers are biologically distinct subgroup of pediatric malignancies with unique clinical characteristics and survival patterns. Evidence from low and middle-income countries (LMICs) remains scarce. This study aimed to evaluate the clinico-epidemiological profile, treatment patterns, and survival outcomes of infant cancers presenting to a tertiary pediatric oncology center in Nepal.

Methods: A retrospective observational cohort at Kanti Children's Hospital, Nepal, including infants diagnosed with malignancy between January 2019 and December 2023. Infants symptomatic before 12 months of age with a confirmed primary malignancy were included. Patients with benign tumors and incomplete medical records were excluded. Demographic and clinical characteristics were analyzed descriptively. Overall survival (OS) and event-free survival (EFS) were estimated using the Kaplan-Meier curve.

Results: Among 134 cases included, 109 were evaluable for survival analysis.63.4% were male and 85.8% were from rural areas. Retinoblastoma (30.6%), Wilms tumor (16.4%), acute lymphoblastic leukemia (14.2%), germ cell tumors (14.2%), and neuroblastoma (11.9%) were common malignancies, with solid tumors accounting for 81.3% of cases. The most frequent presenting symptom was swelling or mass (57.5%). 47% experienced diagnostic delay with a mean time to diagnosis of approximately 81 days. Treatment abandonment occurred in 14.2% of cases. The overall survival was 79.1% (95% CI: 69.0%-84.9%) at 1 year and 71.6% (61.7%-79.4%) at 2 years. Survival was markedly higher in solid tumors [1-year OS 87.4% (77.0%-91.9%); 2-year OS 79.6% (69.2%-86.8%)] compared with hematological malignancies [(1-year OS 38.9% (17.5% -60.0%); 2-year OS 32.4% (12.7% -54.0%)]. Overall EFS was 76.9% (66.7%-82.9%) at 1 year and 65.8% (55.8%-74.0%) at 2 years.

Conclusion: Solid tumors, particularly retinoblastoma and Wilms tumor were common infant cancers. Despite relatively favorable survival for solid tumors, hematological malignancies show poor outcomes. Diagnostic delay and treatment abandonment remain major challenges. Strengthening early referral systems, supportive care infrastructure, and pediatric oncology services is essential to improve outcomes for infant cancers in low-resource settings.

Abstract 386

Performance of Artificial Intelligence Models in Clinical Questions on Hodgkin Lymphoma: A Multicenter Comparative Analysis

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Purpose: Artificial intelligence-based large language models (LLMs) are emerging as potential decision-support tools in oncology. However, their clinical reliability and performance stability under increasing question complexity remain unclear. This study evaluated the performance and clinical utility of different LLMs in responding to clinical queries regarding Hodgkin Lymphoma (HL).

Methods: Thirty open-ended clinical questions on HL were processed by three LLMs: ChatGPT, Claude, and Gemini. Responses were evaluated by six pediatric oncology experts from three centers using a 5-point Likert scale across nine dimensions: accuracy, understandability, clarity, consistency, hallucination, clinical risk, source suitability, professional tone, and up-to-dateness. Statistical analysis was performed using Generalized Estimating Equations (GEE) models.

Results: The "completely accurate" response rates were 42.9% for Claude, 31.7% for Gemini, and 25.4% for ChatGPT. In terms of evidence-based support, Claude achieved the highest "completely suitable" source rating (37.3%), while 46.3% of Gemini's references were only "partially suitable." Up-to-dateness scores were relatively homogeneous but limited across all models (range: 32.3%–35.4%). Multivariate analysis revealed that model performance varied significantly with question difficulty ($p<0.001$). For high-difficulty questions, Claude demonstrated a significantly higher probability of superior performance compared to other models, with Odds Ratios (OR) of 7.59 for understandability (OR=7.597), 3.96 for accuracy (OR=3.962), and 3.75 for clarity (OR=3.752). In contrast, Gemini showed no significant performance shift as difficulty increased ($p>0.05$).

Conclusion: While LLMs provide a baseline of clinical information, they lack consistent reliability in accuracy, safety, and source integrity as clinical complexity increases. Given the zero-tolerance for error in pediatric oncology, these systems are currently insufficient as standalone clinical tools. AI-generated content should only serve as a supplementary resource under the strict supervision of experienced pediatric oncologists and must not replace expert clinical reasoning.

Abstract 387

Chinese Expert Consensus on Early Detection Early Diagnosis and Early Treatment of Children Cancers

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Purpose: To pool the intellectual resources of Chinese pediatric cancer experts, investigate, evaluate, and formulate expert consensus on early diagnosis and treatment of childhood cancer, thereby promoting the prevention and control level of childhood malignancies in China.

Methods: The secretariat team conducted international literature retrieval and review. They convened Chinese pediatric cancer experts for consultation and discussion, carried out questionnaires and opinion analysis among patient families, and ultimately formulated an expert consensus on early diagnosis and treatment of pediatric tumors.

Results: Consistent with international literature, early diagnosis and treatment of pediatric tumors focus on comprehensive prevention and control strategies, primarily divided into three levels: first, social science popularization education to enhance public awareness, enabling early detection through parent-based home self-examination; second, professional science popularization education to improve the awareness of primary healthcare and medical institutions, facilitating early diagnosis; third, establishing a reasonable and scientific professional treatment network to provide higher-level comprehensive treatment at referral centers.

Conclusion: Early diagnosis and treatment of pediatric tumors in China hold significant importance and align with the WHO's GICC policy on childhood cancer. Effective collaboration across these three levels will effectively improve the prevention and control level of childhood tumors.

Abstract 388

Respiratory Viral Infections in Paediatric Oncology: Spectrum, Antiviral Use, and Outcomes in Non-transplant Setting

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Purpose: Immunosuppression from chemotherapy and the underlying malignancy increases susceptibility to viral respiratory tract infections (RTIs) and their complications. Data from South Asia in non-transplant paediatric oncology settings remain limited

Methods: We retrospectively analysed 112 children aged ≤15 years admitted with viral RTI (January–September 2025). Nasopharyngeal and non-bronchoscopic bronchoalveolar lavage multiplex PCR sampling were performed. All patients with febrile neutropenia received intravenous antibiotics per institutional protocol. Descriptive statistics were used for categorical variables and logistic regression for odds ratio calculation.

Results: Median age was 5.0 years (IQR 3.8–8.0); 77.7% had haematological malignancies, 94.6% were on intensive therapy, and 63.4% had prior corticosteroid exposure. Median ALC, ANC, and haemoglobin were 0.8 (IQR 0.3–2.0) $\times 10^9$/L, 0.4 (IQR 0.08–3.6) $\times 10^9$/L, and 8.6 (IQR 7.5–9.8) g/dL. At presentation, 10.7% (n=12) had hypoxia (SpO_2 <94%); 20.5% (n=23) required ICU care, 9.8% (n=11) required mechanical ventilation, and 8.9% (n=10) developed MODS. Median hospitalisation was 8.0 days (IQR 5.0–18.0). Thirty-day and 60-day mortality were 6.3% (n=7) and 8.0% (n=9) respectively. Rhinovirus was the most prevalent pathogen (42.9%, n=48; ICU 27%; mortality 10.4%), followed by Influenza A (21.4%, n=24; ICU 4%; mortality 8.3%), RSV (15.2%, n=17; ICU 29%; mortality 11.8%), Coronavirus (8.0%, n=9; ICU 11%), Parainfluenza (4.5%, n=5; ICU 40%), and Adenovirus (3.6%, n=4). Antivirals were administered in 87/112 (77.7%) — oseltamivir (n=70), ribavirin (n=36), and combination therapy (n=21). IV antibiotics were used in 96.4%; proven bacterial pneumonia occurred in 4.5%, exclusively in ICU-admitted patients. Independent predictors of death were MODS (OR 45.2; 95% CI 8.9–229.6; p<0.001), bacterial coinfection (OR 16.3; 95% CI 2.4–110.8; p=0.004), and prolonged hospitalisation (p=0.01).

Conclusion: Viral RTIs cause significant morbidity and mortality in paediatric oncology patients, particularly those with MODS and bacterial coinfection, but many are salvageable with early antiviral and supportive care. Multiplex PCR-guided diagnosis can optimise antimicrobial and antiviral stewardship.

Abstract 389

Reducing Childhood Cancer Treatment Abandonment Through a Bundled Psychosocial Support Program in Indonesia: A Mixed-Methods Implementation Study

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Purpose: Treatment abandonment remains a major barrier to improving childhood cancer survival in LMIC, including Indonesia, where rates may reach approximately 20%. Abandonment is driven by psychosocial, financial, and health-system barriers that affect families during cancer treatment. Context-responsive strategies integrated into routine care are needed to identify and support families at risk.

Methods: We conducted a mixed-methods implementation study at a tertiary pediatric oncology center in Indonesia between January 2024-December 2025. Abandon rates were compared among children diagnosed in 2024 and 2025. In parallel, semi-structured interviews were conducted with caregivers of children who abandoned treatment to explore barriers to care. Qualitative findings were analyzed concurrently and used iteratively to refine a bundled psychosocial support intervention integrated into routine clinical workflows. The intervention bundle included five components: structured psychosocial risk assessment; access-to-care support including logistical and referral coordination; health literacy and caregiver counseling; linkage to socioeconomic assistance and charitable resources; and system navigation with patient advocacy to facilitate treatment continuity.

Results: A total of 281 children diagnosed in 2024 and 259 children diagnosed in 2025 were included. Treatment abandonment decreased from 11.7% (33/281) in 2024 to 7.3% (19/259) in 2025, corresponding to an absolute reduction of 4.4% and a relative reduction of 37.6%. This represents a clinically meaningful decline, although the difference did not reach statistical significance (p = 0.083). During implementation, 369 families received at least one component of the psychosocial support bundle, with many receiving multiple interventions. The most frequent components were psychosocial risk assessment, caregiver counseling, and socioeconomic support. Qualitative interviews with 23 caregivers identified drivers of abandonment including financial hardship, emotional distress, limited treatment understanding, and logistical barriers such as transportation and accommodation. These findings informed iterative refinement of the psychosocial support bundle.

Conclusion: A context-adapted bundled psychosocial support intervention was promising strategy for reducing treatment abandonement in LMIC

Abstract 390

Implementation of a Pediatric Total Body Irradiation Program using a Translational Couch Technique: Initial clinical experience in Qatar

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Purpose: Total body irradiation (TBI) remains an important component of conditioning regimens for allogeneic hematopoietic stem cell transplantation (HSCT) in children. This study reports the implementation of a pediatric TBI program at the National Center for Cancer Care and Research, Hamad Medical Corporation (NCCCR-HMC), Doha, Qatar.

Methods: Building upon established institutional expertise with translational couch TBI in adults, we adapted the technique for children. Patients are positioned sequentially supine then prone on a computer-controlled motorized couch on the floor, moving translationally across a vertically oriented photon beam at an extended source-to-skin distance through Perspex beam spoiler. Couch velocity is modulated to compensate for patient thickness variations. Partial transmission

blocks reduce lung and kidney doses, with supplemental electron boosts to shielded regions to restore dose homogeneity. A multi-isocenter VMAT-based TBI technique was concurrently commissioned as a backup for anesthesia-dependent younger patients.

Results: Three pediatric patients with relapsed B-cell acute lymphoblastic leukemia (ALL) underwent TBI-based conditioning prior to HSCT between October 2025 and March 2026. All were treated using the translational couch technique and received 12 Gy in 6 fractions, delivered twice daily over three consecutive days. All treatments were completed successfully without interruptions. Beam-on time per fraction ranged from 28 to 32 minutes. Acute toxicities were mild: Patient 1 (12-year-old girl, matched sibling HSCT) experienced grade 1 oral mucositis and transient diarrhea; Patient 2 (13-year-old girl, haploidentical HSCT, prior cranial irradiation) developed mild hemorrhagic cystitis and anemia; Patient 3 (9-year-old boy, matched sibling HSCT) had grade I cutaneous acute graft-versus-host disease following HSCT. All patients achieved successful engraftment and satisfactory immune reconstitution.

Conclusion: The introduction of pediatric TBI using a translational couch technique at our institution proved feasible and safe. Treatment duration remained acceptable and therapy was well tolerated, likely reflecting the older age group and comprehensive psychosocial preparation of children.

Abstract 391

Clinically Feasible Integrated Molecular Stratification Identifies Prognostic Subgroups in Standard-Risk Medulloblastoma

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Purpose: Although several molecular stratification systems for medulloblastoma have been proposed, their implementation is limited. The aim was to develop a clinically feasible molecular stratification algorithm for patients with standard-risk medulloblastoma.

Methods: The study included 134 patients with standard-risk medulloblastoma (age >3-5 years, R0M0, non-anaplastic histology, absence of MYC/MYCN amplification) treated between 2012 and 2024. Molecular subgroup assignment was performed using NanoString nCounter expression profiling (23-gene panel). Mutation assessment was performed only in WNT (n=33) and SHH (n=17) subgroups. All patients received craniospinal irradiation at a dose of 23.4–35.2 Gy with a posterior fossa boost to 54–55 Gy followed by maintenance chemotherapy.

Results: The distribution of patients across molecular subgroups was as follows: WNT (n=41), SHH (n=20), Group 3 (n=23), and Group 4 (n=50). WNT tumors demonstrated the most favorable outcomes, with 5-year overall survival (OS) of 93% and event-free survival (EFS) of 84%. In contrast, survival was lower in the SHH and Group 3 (OS 77% and 74%; EFS 60% and 63%, respectively). Based on expression profiling, tumors within the non-WNT/non-SHH cohort were further stratified into three transcriptional subtypes: GR3-pure, GR4-pure, and GR3/4. The poorest 4-year EFS was observed in the GR3-pure (67%) and GR3/4 (57%) subtypes, whereas the GR4-pure subtype was associated with a favorable outcome (87%, p=0.023). TP53 mutations were identified in 24.2% of WNT and 29.4% of SHH tumors and were associated with inferior 3-year EFS (p<0.020). The proposed molecular stratification algorithm identified two prognostic groups with significantly different survival outcomes (p<0.001): the 3-year EFS was 62% in the unfavorable group and 87% in the favorable group.

Conclusion: These findings demonstrate substantial biological heterogeneity within standard-risk medulloblastoma. The proposed molecular stratification algorithm enables identification of prognostically unfavorable disease variants and may provide a practical framework for future risk-adapted therapeutic strategies.

Abstract 392

Decreased postural control in survivors of childhood cancer

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Purpose: The toxicity of anticancer therapies leads to a range of musculoskeletal issues. However, there is a lack of research on the clinical presentation of postural disorders in pediatric and adolescent cancer patients. Aim of the study was to evaluate the prevalence of postural disorders as predictors of diseases of the musculoskeletal system among children who had completed treatment for cancer.

Methods: Single-stage cross-sectional research. Physical status included: anthropometry, the development of basic physical qualities, body composition, and posture characteristics using the KOMOT method. When comparing the observed proportion of the trait with the reference proportion, the Z-criterion for one proportion was used. The values of 25% and 75% of the standard group were used as reference proportions. The relationship between the variables of posture and muscles endurance was calculated by the method of canonical correlation analysis.

Results: Totally of 1,492 children and adolescents (aged 6–18 years) were enrolled in the study. The experimental group consisted of 892 pediatric cancer survivors in remission (54.5% male; mean age 11.5 ± 3.3 years; mean time since treatment 4.6 ± 2.5 years). The control group included 600 healthy peers from six Russian regions (50.0% male; mean age 11.8 ± 3.3 years). Significant postural abnormalities were observed in the survivor group ($p < 0.001$), including: relative spinal shortening (31%), increased thoracic kyphosis (31.8%), shoulder tilt (32.3%), trunk lean (34.0%), pelvic rotation in the horizontal plane (30.4%), and scoliotic posture (42.5%). Canonical correlation analysis revealed moderate positive associations between postural parameters and both muscular endurance ($rcc = 0.425$, $p < 0.001$) and the skeletal muscle mass (SMM) percentage ($rcc = 0.650$, $p < 0.001$).

Conclusion: Pediatric cancer survivors exhibit a significantly higher prevalence of postural impairments compared to healthy peers ($p < 0.001$). The moderate positive correlation between postural parameters, muscular endurance, and skeletal muscle mass suggests that secondary musculoskeletal complications in this population are closely linked to sarcopenia and reduced physical fitness. Rehabilitation programs for survivors should prioritize strength training and body composition optimization to mitigate long-term postural deficits.

Abstract 393

CAREGIVERS' EVALUATION OF THE TELEMEDICINE SYSTEM USED IN A TERTIARY PEDIATRIC CANCER CENTER IN A LOW-RESOURCE COUNTRY

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Purpose: The Aruga Telemedicine is the only telehealth system in the Philippines dedicated for children with cancer and hematologic conditions. Since gaining traction during the recent pandemic, its use has become a major strategy to improve healthcare delivery and navigation at reduced costs. However, no study has been conducted to evaluate its performance. In addition, there is paucity of studies on the use of telehealth among pediatric hematology and oncology patients in the country. Thus, this study was done to evaluate the Aruga Telemedicine system among caregivers in terms of the National Quality Forum (NQF) domains for assessing teleheath: access to care, cost, experience, and effectiveness. The study also aimed to describe the relationship between respondent evaluation and their sociodemographic profiles and the physical setting of the consultations.

Methods: The study was a quantitative relational research conducted using a researcher-developed, self-administered questionnaire with two (2) parts: (I) Respondent Characteristics and (II) Performance Evaluation. The frequency of each variable and the scores for the evaluation were then tallied and statistically analyzed for possible association.

Results: Among the 149 respondents, the Aruga Telemedicine received mostly favorable ratings across the NQF domains, gaining an average score of 88.6 out of 98 points per respondent. The factors found to influence the respondents' evaluation are the patient's sex and region of residence, the caregiver's educational attainment, the part or area of the house used for the teleconsultation, and both the main internet source and perceived internet strength. Strong linear correlations were also found between the individual NQF domains.

Conclusion: The results of the study indicated a positive perception among caregivers towards the performance of the Aruga Telemedicine system across the NQF domains. The overall rating was high among the caregivers of patients, demonstrating the system's usefulness and acceptability across sociodemographic

characteristics and technological resources.

Abstract 394

Age at cancer diagnosis determines the presence and severity of dental developmental disturbances in childhood cancer survivors

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Purpose: Childhood cancer survivors are at increased risk of dental developmental disturbances; however, determinants of both their occurrence and severity remain incompletely understood. This study aimed to identify age- and treatment-related risk factors for dental developmental disturbances using both binary and continuous outcome measures.

Methods: We conducted a retrospective cohort study of 97 childhood cancer survivors treated at a single institution. Dental developmental disturbances were evaluated using a binary outcome (presence or absence) and the Modified Dental Developmental Index (MDDI) as a continuous measure of severity. Multivariable logistic regression was used to identify factors associated with the presence of disturbances, and multivariable linear regression with bootstrap confidence intervals was used to assess factors associated with MDDI severity. Models included age at diagnosis, radiotherapy, cumulative alkylating agent dose (CED1000), hematopoietic stem cell transplantation (HSCT), and sex. Sensitivity analyses were performed to assess the robustness of the findings.

Results: Younger age at diagnosis was the strongest independent predictor of dental developmental disturbances. Compared with patients diagnosed before age 3 years, those diagnosed at ≥10 years had a significantly lower risk (adjusted odds ratio 0.05, 95% confidence interval 0.01–0.31). Radiotherapy was also associated with a lower risk after adjustment for age and treatment-related factors. In multivariable linear regression analysis, younger age at diagnosis was independently associated with higher MDDI scores, indicating greater severity (B −0.055 per year, 95% bootstrap confidence interval −0.085 to −0.028). HSCT was associated with increased MDDI severity, whereas cumulative alkylating agent dose and sex were not independently associated. Sensitivity analyses yielded consistent results.

Conclusion: Younger age at cancer diagnosis is a key determinant of both the presence and severity of dental developmental disturbances in childhood cancer survivors. HSCT was associated with greater severity. These findings highlight the importance of age- and treatment-specific dental surveillance in survivorship care.

Abstract 395

Adherence to the Clinical Practice Guidelines for Diagnosing and Managing Pediatric Acute Lymphoblastic Leukemia in a Tertiary Hospital

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Purpose: Acute lymphoblastic leukemia (ALL) is the most common childhood cancer, peaking at ages 2-5. Despite advances, survival rates in the Philippines are lower than in high-income countries, partly due to uneven care access. Clinical practice guidelines (CPGs) were developed for evidence-based pediatric ALL management. The Children's Cancer Institute of Southern Philippines Medical Center (SPMC) adopted a CPG for diagnosis and treatment. This study evaluated adherence to these guidelines in clinical assessment, diagnostics, risk stratification, and treatment for newly diagnosed children and adolescents.

Methods: A retrospective chart review was done at SPMC in Davao City, examining records of patients aged 0–18 with newly diagnosed ALL at least six months after the CPG's release. Total sampling was used. Data from a chart audit assessed guideline compliance on clinical assessment, diagnostics, risk stratification per NCI criteria, and treatment initiation. Descriptive stats summarized demographics and compliance rates.

Results: Forty charts were reviewed. The average age was 6.37 years, mostly 0–5 years (57.5%) and male (72.5%). Common symptoms included lymphadenopathy (92.5%), fever (82.5%), pallor (72.5%), hepatomegaly (65%), and bone pain (50%). Bone marrow aspiration with flow cytometry was done in 72.5% of cases. Risk stratification based on age, white blood cell count, and extramedullary disease was documented in all cases. Compliance showed 100% adherence for history taking and risk stratification, 90% for bone marrow aspiration and flow cytometry when available, and 85% for treatment protocols.

Conclusion: Adherence to pediatric ALL guidelines was generally high in this hospital, especially in assessment and diagnostics. However, compliance with treatment protocols was slightly lower, possibly due to resource barriers or variability. Ongoing monitoring, education, and quality improvement are needed to enhance guideline use and improve pediatric oncology care.

Abstract 396

Beyond Deauville: Improving Response Evaluation in Multiple Myeloma with IMPeTUs Criteria

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Purpose: Multiple myeloma (MM) is a heterogeneous disease characterized by focal bone lesions and extramedullary manifestations. ^{18}F-FDG PET/CT is an imaging method that allows assessment of disease extent and metabolic activity of lesions throughout the body, including evaluation of lytic bone lesions. The aim of this study was to identify the limitations of the DS in MM and propose an alternative scale for clinical trials and routine practice.

Methods: A literature-based comparison of IMWG criteria, the IMPeTUs scale, and the Deauville Scale (DS) was conducted.

Results: The IMWG criteria do not include the DS but assess MM considering laboratory markers, bone marrow morphology, and imaging of bone lesions via radiography, CT, MRI, and PET/CT. The use of the DS in MM may yield ambiguous results in evaluating disease activity due to low hexokinase-2 expression, which plays a key role in ^{18}F-FDG phosphorylation and subsequent accumulation in tumor cells, potentially leading to false-negative ^{18}F-FDG PET/CT findings. The visual IMPeTUs criteria include key radiological patterns in MM, such as bone marrow activity, foci of radiotracer hyperfixation, focal bone lysis, fractures, and extramedullary involvement. This scale can complement IMWG criteria with specific imaging findings.

Conclusion: The use of the DS for assessing MM treatment response is controversial due to differences in biology (bone lesions) and response criteria (consideration of monoclonal protein). The DS does not allow prognosis assessment based on metabolic activity levels and is not recommended in IMWG international criteria for routine ^{18}F-FDG PET/CT studies. A qualitative visual assessment is needed instead of a numerical scale, focusing on the dynamics of specific lesion activity. The IMPeTUs scale is an optimal alternative, reflecting radiological patterns of MM and integrable with IMWG criteria. Prospective studies are required to validate IMPeTUs, assess reproducibility, and determine prognostic significance.

Abstract 397

Pain in children with cancer, a single institution experience.

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Purpose: Perception of pain among children with cancer is complex, these children commonly experience pain throughout their disease and cancer therapy, significantly affecting their quality of life. However, studies reveal that pain management in paediatric oncology settings remains suboptimal with significant gaps in the use of standardised pain assessment tools and guidelines. Therefore, this study explores the prevalence and management of pain in children with cancer at the Paediatric Haematology & Oncology Unit at UKM Specialist Children's Hospital.

Methods: A cross-sectional study was conducted among children aged 2 to 18 years old on active cancer treatment, excluding those on palliative care. A content validated questionnaire assessing pain experience, severity, characteristics and pain management was self administered for children aged 12 years old and above, whereby younger children were guided by parents/guardians. Respondents' diagnosis and treatment phases were obtained from the hospital electronic medical records.

Results: A total of 118 respondents participated in this study, nearly half (47.5%) reported experienced pain in the past 7 days with a median pain score of 4. Cancer

related pain (37.5%) was the most prevalent followed by procedural pain such as branula insertion (28.6%), bone marrow sampling (12.5%) and intrathecal procedure (8.9%). Pain score was assessed in inpatient for 46.6% of the respondents. Those who had pain without pain score assessed reporting a significant higher pain score. Paracetamol was the most frequently used pharmacological treatment (51.7%), while non-pharmocological measures, such as rest (78%), position change (28.8%), and massage (28.8%) were widely adopted to relieve pain. Most patients (97.4%) were satisfied with the pain treatment and 96.6% reported hospital staff responded quickly towards pain complaints.

Conclusion: The study higlighted significant gaps in pain assessment and management, emphasise the need for pain assessment protocol and family-centered approaches to enhance paediatric pain management.

Abstract 398

Clinical Outcome Evaluation of Diagnostic Concordance Between In-Hospital Pathology Reports and External Pathology Review of Pediatric Cancer Patients: A 4-Year Retrospective Study in a Resource-Limited Setting

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Purpose: Accurate histopathological diagnosis is the foundation of effective pediatric oncology. In resource-limited settings, limited access to subspecialized pediatric pathology can lead to significant diagnostic disparities. This study assessed the concordance between institutional pathology reports and external reviews by an international partner and evaluates the subsequent impact on clinical management and patient outcomes.

Methods: A retrospective study was conducted on pediatric patients (age <19 years) at a regional tertiary center whose tissue specimens were sent to an international partner for secondary review between 2016 and 2019. Diagnostic concordance was categorized as full agreement, partial agreement, or discordance. Clinical outcomes and treatment modifications resulting from these reviews were analyzed.

Results: A total of 140 pediatric patients were included in the study, with mean age of 10.75 years and male predominance (76/140, 54.29%). Full diagnostic concordance was achieved in 38.57% (n=54), partial agreement in 37.14% (n=52), and discordance in 24.29% (n=34). The majority of discordant cases (91.18%) were malignant, with solid tumors (55.88%) being more frequent than hematologic malignancies (35.29%). External review led to treatment modification in 33.57% (n=47) of the total cohort. Of the patients alive during the study period, 72.55% had concordant results. Among those who died, 80.36% had concordant initial results and 19.64% had discordance. This suggests that while discordance allows for salvage therapy, many concordant cases represent inherently high-risk disease.

Conclusion: One in three children required a change in clinical management following an external pathology review. The most significant element causing diagnosis disparities is the lack of modern molecular diagnostic methods. This diagnostic gap underscores the critical role of international "twinning" partnerships and the urgent need for investment in modern molecular diagnostic facilities within regional centers to bridge survival disparities in low- and middle-income countries.

Abstract 399

Initiatives to enhance pneumonia outcomes in pediatric oncology patients through the application of high-flow nasal cannulas in regular wards: A Pilot Quality Improvement Initiative

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Purpose: Pediatric Intensive Care Unit (PICU) bed capacity in many developing countries is limited, and oncology patients are not a priority for admission. Furthermore, oncology patients are at higher risk of infection, particularly in the respiratory tract. Recommendations indicate that high-flow nasal cannula (HFNC) can be commenced in the initial phases of respiratory distress, potentially preventing unfavorable outcomes such as emergency intubation and mortality. In our setting, the use of HFNC was initially limited in the PICU setting, but given the need to assist a wider range of patients, we initiated its use in non-intensive settings. Training was conducted for attending physicians and nurses before implementing HFNC in our regular wards.

Methods: This was an interventional study as a part of a quality improvement project performed at a tertiary-academic hospital. Oncology patients admitted with a diagnosis of pneumonia to the ward and experiencing mild to moderate respiratory distress were included. Implementation of an HFNC protocol started on January 1st, 2025. The primary outcome was emergency intubation, secondary outcome was mortality rate.

Results: We enrolled 9 patients during a 1-year period. Patient demographics showed the median age was 7.3 years old, with a dominantly male patient (56%). The most common malignancy diagnosis was acute lymphoblastic leukemia. The initial settings used were a mean flow of 1.3 liters per kgBW per minute and a mean fraction of inspired oxygen (FiO_2) of 0.45. The mean difference of heart rate (HR), respiratory rate (RR), and peripheral saturation pre- and 1-hour post-application of HFNC was 4x/minute, 3x/minute, and 3%, respectively. The rate of emergency intubation was 22% (2/9) and the mortality rate was 33% (3/9).

Conclusion: The application of HFNC in the ward indicates it to be promising in reducing mortality from pneumonia in pediatric patients with malignancies who are ineligible for PICU admission. More subjects are still needed to prove the effectiveness of this initiative.

Abstract 400

Integrated Approach to Establishment of Neurosurgical Service at the Scientific-Practical Medical Center for Pediatric Oncology, Hematology and Immunology (Tashkent, Uzbekistan): From Organizational Decisions to First Operations

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Purpose: Creation of a pediatric neurosurgical service de novo within an oncology center in Uzbekistan faces infrastructural limitations. The priority is establishing clinical governance and multidisciplinary decision-making.

Methods: Description of organizational model and analysis of activities over six months from October 2025 to March 2026 based on the SPMCPOHI (Tashkent, Uzbekistan). Components of work: Normative-methodical base: Development of 12 standard operating procedures (SOPs) and checklists Multidisciplinary consultation (Tumor Board): Weekly neuro-oncology tumor board meetings. Protocols of decisions are documented and serve as a basis for therapy adjustments. CNS Tumor Registry Clinical Work: Patient consultations across all departments of the center; performing urgent neurosurgical interventions.

Results: Documentation: Implementation of 12 SOPs standardized staff actions in case of neurological complications and reduced time to neurosurgical intervention. Tumor Boards: Since December 2025, six sessions have been conducted reviewing 18 clinical cases. In 77.78% of cases, tumor board recommendations led to changes in therapeutic strategy CNS Tumor Registry: Fifty patients with various diagnoses Consultation and Surgery: More than 100 children were consulted. Four urgent surgeries performed

Conclusion: Establishing a neurosurgical service in an oncology center is a process beginning long before the first operation. This experience can serve as a roadmap for other Asian centers in their formative phases.

Abstract 401

OVERCOMING BARRIERS IN TREATMENT ADHERENCE OF PEDIATRIC PATIENTS WITH CANCER: EXPERIENCE FROM A TERTIARY CANCER CARE CENTRE FROM NORTH-EAST INDIA

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Purpose: Treatment Abandonment (TA) remains a major cause of lower survival among pediatric patients with cancer in low and middle income countries. This study aims to assess prevalence and contributing factors of TA in a tertiary care

centre and compare findings with previously published data.

Methods: Data were collected from hospital database for all patients under 18 years of age with histopathologically or cytologically proved malignancy, registered from January 2021 to December 2024 (Study A). Findings were compared with previously published data from the same centre (Asian Pac J Cancer Prev. 2019 Apr 29;20(4):1133-1137) prior to implementation of patient support programs with patients registered between April 2010 and March 2017 (Study B)

Results: In Study A, 231 of 1,865 patients (12.3%) abandoned treatment compared to 161 of 592 patients (31%) in Study B. Significant predictors of TA in both cohorts included place of residence (urban: Study A: Odds Ratio [OR] = 0.78, 95%CI 0.251-2.453, Study B:OR = 0.83, 95% CI 0.565-1.228); maternal education (Study A p = 0.005, Study B p=0.505); financial constraints (Study A: 5.7%, Study B: 28.6%) and long travel distances (Study-A: 81.2%; Study-B:12.4%). Females had higher risk of TA (Study A OR female = 1.14, 95%CI 0.400-1.355, Study-B OR male = 0.70, 95% CI 0.483-1.018), In Study-A additional factors for TA were caregiver's belief in incurability (66.1%) and ineligibility for financial assistance schemes (p = 0.038). The highest abandonment rates in Study-A were observed in Acute Myeloid Leukemia (40%), followed by Neuroblastoma (20%), Acute Lymphoblastic Leukemia (12%), Brain Tumors (9%) and Bone/Soft Tissue Sarcomas (7%).

Conclusion: Financial constraints, maternal education, long travel distance, belief in incurability are major causes of TA. Implementation of active patient support programs including financial, nutritional, psychological, and accommodation assistance since 2019 have significantly reduced TA. Targeted interventions can further improve treatment adherence in childhood cancer.

Abstract 402

Epidemiology of malignant neoplasms in young children in the Kyrgyz Republic

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Purpose: Malignant neoplasms (MN) found in young children often have clinical and biological properties that differ from the properties of the same histological types of MN found in older children. The purpose: study the aspects of descriptive epidemiology of malignant tumors in young children in Kyrgyzstan.

Methods: Over 15 years 517 cases of MN were registered in children in the age group (0-4 years). Age-standardized incidence rates calculated (world standard), depending on gender, age, ethnic group, and region. Annual number of children based on the materials of the National Statistical Committee were used.

Results: Leukemias were in the first place - 172 (33%). The incidence rate in the urban population was 85.7, and in the rural - 54.8 (p = 0.023). There was a statistically significant increase in the incidence of MN in children of the Russian ethnic group (121.7) compared with other nationalities.

Conclusion: In the younger age group, the incidence of cancer is higher than in middle and older childhood, but relatively lower than in developed countries. It is necessary to further study the features of the prevalence of tumors in children in Kyrgyzstan using large groups and analytical methods of epidemiology.

Abstract 403

Infection Prevention (MCIP) Effectiveness of a Mother Centered in Reducing Educational Intervention on Infection Prevention Infections among Oncology Pediatric Patients: A Randomized Controlled Trial

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Purpose: Infection-related morbidity and mortality remain major barriers to successful pediatric cancer treatment in LMICs. Caregiver education represents a feasible, low-cost strategy to strengthen supportive care and prevent avoidable infections.

Methods: A randomized controlled trial was conducted at PIMS Islamabad including 80 mothers of pediatric cancer patients. Participants were randomized to intervention or control groups. The intervention included five weekly educational sessions, teach-back, IEC materials, and reinforcement visits.

Results: Mean maternal KAP scores increased by 52% in the intervention group versus 11% in controls (p<0.001). Infection-related hospitalizations (10% vs 45%) and antibiotic use (12.5% vs 57.5%) were significantly reduced in the intervention group.

Conclusion: This Health Belief Model–based, mother-centered intervention significantly improved caregiver competencies and reduced infection-related outcomes. It is a feasible, culturally appropriate, and scalable supportive care model for pediatric oncology in resource-limited settings.

Abstract 404

Breaking the Chain: Successful Control of a Candidemia Outbreak in a Jordanian Pediatric Oncology center .

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Purpose: Pediatric oncology patients face high candidemia risk from chemotherapy-induced neutropenia, mucosal damage, central lines, broad-spectrum antibiotics, and ICU care. Resistant strains drive >20% mortality despite antifungal advances.

Methods: This case series describes a non-C. auris candidemia outbreak at KHCC (June–Sep 2025) in pediatric oncology patients, defined by positive blood/CSF cultures or suggestive imaging.

Results: Of 12 cases: 75% leukemia (4AML/2B-ALL/1T/rT/rB-ALL), 17% glioma, 8% histio. 100% lines, 92% abx, 58% PICU. C.trop 41%; others 8%; ID 33%. AzoleR 40%. Normal eyes. Inv:67% (hep42%, pulm33%). KHCC: surv/stew/cohort/hyg/isol/vis/proph/pause chemo. IDSA ech/amph ≥14d: 66% surv despite R; 1/12 C death

Conclusion: Despite 40% resistance, comprehensive control yielded 8% Candida-attributable mortality (vs. >20% literature), exemplifying multidisciplinary management in a high-risk LMIC pediatric cohort.

Abstract 405

Leveraging Capacity Building to Improve Pediatric Oncology Care in Kenya: Experience From Pediatric Oncology Training Workshops in Western Kenya

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Purpose: Childhood cancer is a growing global health challenge, with approximately 400,000 new cases diagnosed annually. Survival exceeds 80% in high-income countries but remains below 30% in many low- and middle-income countries (LMICs), largely due to delayed diagnosis, limited specialized workforce, and weak referral systems. In Kenya, low awareness among frontline healthcare workers contributes significantly to diagnostic delays and poor outcomes.

Methods: We implemented pediatric oncology training workshops in Eldoret, Kenya, through a multidisciplinary collaboration involving Moi University, Moi Teaching and Referral Hospital (MTRH), Indiana University (USA), and the Princess Máxima Center (Netherlands). Participants were selected to ensure geographic and professional diversity. Training included lectures, case-based discussions, and interactive group work. Attendance data and participant feedback were collected.

Results: Between 2019 and 2026 (excluding 2021), seven workshops trained 733 healthcare workers across multiple cadres. Participation increased substantially following the introduction of hybrid learning models, with 184 participants in both 2025 and 2026, including a growing proportion of virtual attendees. Participants reported improved confidence in recognizing childhood cancer signs and understanding referral pathways. Participating facilities also reported increased referrals of suspected cancer cases.

Conclusion: Pediatric oncology training workshops are a scalable and impactful strategy for improving early cancer detection in LMICs. The adoption of blended learning enhances reach and sustainability. However, training alone is insufficient; strengthening health systems, diagnostic capacity, and access to treatment remains essential to improving survival outcomes.

Abstract 406

Implementation of a Structured Early Cardio-Oncology Monitoring Algorithm for Children Receiving Anthracycline Therapy: Initial Clinical Experience

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Purpose: Anthracycline-based chemotherapy in children carries a risk of both early and late cardiotoxicity. Subclinical myocardial injury often remains undetected by conventional monitoring, as left ventricular ejection fraction (LVEF) may be preserved despite early cardiotoxic damage. Current cardio-oncology guidelines - including position statements from the European Society of Cardiology (ESC) and specialized Eurasian professional societies - stress the need for standardized cardiovascular monitoring in pediatric patients, incorporating both cardiac biomarkers and echocardiography. To develop and implement a structured early cardio-oncology monitoring algorithm for children undergoing anthracycline-based chemotherapy and to report the initial clinical experience from the Scientific and Practical Medical Center for Pediatric Oncology, Hematology and Immunology.

Methods: A prospective inpatient monitoring program was launched at the Scientific and Practical Medical Center for Pediatric Oncology, Hematology, and Immunology. The algorithm involves a baseline cardiovascular assessment (clinical exam, ECG, echocardiography with LVEF assessment, troponin, ST2, and NT-proBNP) followed by stepwise biomarker monitoring at predefined cumulative anthracycline doses. Repeat echocardiographic evaluations were performed throughout treatment, aligned with ESC guidelines adapted for pediatric use. Cases showing dynamic biomarker elevation despite preserved LVEF were reviewed through structured multidisciplinary discussions involving pediatric oncologists and cardiologists.

Results: During the initial phase, a subset of the enrolled patients exhibited sequential increases in cardiac biomarkers despite maintaining a preserved LVEF. These findings prompted intensified surveillance and early multidisciplinary discussions regarding oncologic strategy and cardioprotective management. While no clinically manifest cardiac dysfunction occurred during short-term follow-up, long-term outcomes are currently being evaluated to determine the prognostic significance of these early biomarker elevations.

Conclusion: Implementing a structured early cardio-oncology monitoring algorithm in pediatric practice is both clinically feasible and practically implementable within a specialized pediatric oncology setting. Serial assessment of specific cardiac biomarkers combined with echocardiography identifies subclinical myocardial stress at a preclinical stage, providing a framework for timely multidisciplinary intervention. Further prospective studies in larger cohorts are necessary to confirm the impact of this strategy on long-term outcomes and to harmonize monitoring protocols across European and Eurasian professional cardio-oncology societies.

Abstract 407

Artificial intelligence in automated assessment of tumor metabolic volume in patients with Hodgkin's lymphoma. Development and training

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Purpose: The diagnosis and treatment of patients with Hodgkin's lymphoma (HL) reflects current trends in modern oncology. One of the goals of the search for additional diagnostic criteria using artificial intelligence (AI) technologies is to modify the protocol of the St. Petersburg group of scientists SPbLH ([S]aint-[P]etersburg group for the study of treatment of [L]ymphoma [H]odgkin in children). Metabolic tumor volume (MTV), calculated from positron emission tomography combined with computed tomography (PET-CT) using 18F-fluorodeoxyglucose (18F-FDG) is one of the interesting parameters. The pilot study aims to evaluate the feasibility of automated methods for MTV assessment using AI technology.

Methods: The study included imaging data from 300 pediatric and young adult patients with HL who underwent primary 18F-FDG PET-CT at the N. N. Petrov National Medical Research Center of Oncology, open sources. The proposed method for determining MTV is based on deep learning techniques and involves training neural network (NN) and automated analysis of PET-CT images using a trained model.

Results: PET-CT studies stored in the DICOM (Digital Imaging and Communications in Medicine) format were converted into three-dimensional arrays of intensity values expressed on the SUV (Standardized Uptake Value) scale. Manual segmentation maps of tumor regions were created by experts and used as reference annotations for NN training. After training the NN automatically processes new scans, performs tumor region segmentation, generates a three-dimensional probability map of tumor tissue presence. The post-processing stage includes artifact filtering and refinement of segmented region boundaries. Finally, the MTV is calculated by summing the volumes of all voxels classified as tumor.

Conclusion: The proposed AI-based approach can improve the reproducibility and efficiency of PET-CT image analysis. The integration of automated MTV calculation into a user interface may allow clinicians to use this parameter during initial patient stratification and planning of risk-adapted therapy.

Abstract 408

Diagnostic stage distribution of neuroblastoma in Kazakhstan: a national cohort (2020–2024)

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Purpose: Neuroblastoma is the most common extracranial solid tumor in children, and stage at diagnosis is a key determinant of survival. Approximately 40–50% of patients in high-income countries present with metastatic disease, whereas higher rates are reported in settings with delayed diagnosis. Data from Central Asia are limited. We aimed to describe the epidemiology and stage distribution of pediatric neuroblastoma in Kazakhstan and assess temporal trends and regional variation in stage at diagnosis.

Methods: We conducted a retrospective cohort study of children diagnosed with neuroblastoma in Kazakhstan between 2020 and 2024 using data from two national pediatric oncology centers providing centralized care. Demographic and clinical variables, including age, sex, region of residence, stage at diagnosis, and year of diagnosis, were analyzed. Staging was performed according to the International Neuroblastoma Staging System (INSS). Stage distribution was evaluated overall and stratified by year and region. Regional variation in stage at diagnosis was assessed using chi-squared testing.

Results: A total of 151 children were included. The median age at diagnosis was 2 years (IQR 1–4), with 55.6% male. The estimated annual incidence based on available data was 0.44 cases per 100,000 children. Overall, 66.2% of patients were diagnosed with stage IV disease, while early-stage disease (stage I–III and 4S) accounted for 31.8%; stage was unknown in 2%. The proportion of stage IV disease remained consistently high across all years (62.2–75%). Regional variation was observed, with stage IV proportions ranging from 57.9% to 100% in regions with ≥5 patients, showing a trend toward statistical significance ($p = 0.055$) A high proportion of patients presented with advanced disease: 66.2% were diagnosed with stage IV neuroblastoma, while early-stage disease (stage I–III and 4S) accounted for 31.8%. Stage was unknown in 2% of cases. The proportion of stage IV disease remained consistently high across all years, ranging from 62.2% to 75% between 2020 and 2024. The number of diagnosed cases increased over time, peaking in 2023. Variation in stage at diagnosis across regions was observed, with stage IV proportions ranging from 57.9% to 100% in regions with ≥5 patients; however, this did not reach statistical significance ($p = 0.055$).

Conclusion: The persistently high proportion of advanced-stage neuroblastoma indicates systemic delays in diagnosis in Kazakhstan. Regional variation may reflect disparities in access to timely care. Strengthening early detection and referral pathways may be critical to improving outcomes.

Abstract 409

Improved Survival for Childhood Cancer in a Tertiary Referral Centre in Malaysia

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Purpose: Outcome of children with cancer in Malaysia is unknown.

Methods: We retrospectively reviewed the outcome of children diagnosed with cancer and treated in Paediatric Institute, Hospital Kuala Lumpur and Hospital Tunku Azizah between January 2016 and December 2023.

Results: There were 1456 cases affecting 1451 patients, with 816 boys and 635 girls (M : F = 1.3 : 1). Four patients with Transient Abnormal Myelopoiesis developed Myeloid Leukemia of Down Syndrome and a patient with Retinoblastoma developed Rhabdomyosarcoma during this period. The commonest cancer is Leukemia [428], followed by Central Nervous System [225], Germ Cell [135], and Eye tumours [124], Neuroblastoma [106], Bone tumours [93], Lymphoma [87], Soft Tissue [64], Kidney and Liver tumours [54 each], Langerhan's Cell Histiocytosis (LCH) [45] and others [36]. 5-year overall survival is 65% with no outcome difference between gender. However, there is significant drop in survival with advancing age at diagnosis (0-1 year old 73%, 1-4 years old 70%, 4-7 years old 64%, 7-12 years old 62%, 12-18 years old 52%). Patients were divided into two 4-year periods (2016-2019, 2020-2023) for comparison. 3-year overall survival improved by 5% (65% to 70%), led by Neuroblastoma (50% to 68%), followed by Lymphoma (66% to 83%), LCH (86 to 96%), kidney tumours (86% to 96%) and Leukemia (66% to 75%).

Conclusion: Improved survival was observed over time in the largest childhood cancer treatment centre in Malaysia. Possible factors contributing to this include better patient stratification, advances in treatment regime, improved multidisciplinary care, supportive care and healthcare infrastructure.

Abstract 410

ORGANIZATION OF REHABILITATION OF PATIENTS WITH ONCOHEMATOLOGICAL DISEASES IN THE REPUBLIC OF BELARUS

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Purpose: Children with cancer and hematological malignancies in high income countries now have >80 % survival rates, but treatment complications can reduce quality of life during and after therapy. To determine patient pathways for rehabilitation during treatment and after completion of treatment.

Methods: Pathways were determined at the Belarusian Research Center for Pediatric Oncology, Hematology, and Immunology, treating children and adolescents, as well as certain categories of young adults (up to 29 years of age).

Results: The treatment and rehabilitation stage is carried out at the Center. The Medical Rehabilitation Department employs: - a rehabilitation physician, - a pediatrician, - a physical rehabilitation specialist, - an occupational therapist, - a therapeutic exercise instructor, - a massage nurse, - physical therapy nurses, - psychologists. Rehabilitation is provided in all departments, including the intensive care department, and the transplant department. An interdisciplinary approach is used to develop an individualized medical rehabilitation plan. The following methods are used: - Physical rehabilitation (exercise therapy, active and passive kinesitherapy, cardio training, mechanotherapy), - Physiotherapy, - Diet therapy, - Herbal medicine, - Occupational therapy, - Psychological counseling. Before starting rehabilitation measures, the patient undergoes initial testing: anthropometry, dynamometry, six-minute walk test, Genchi test, Stange test; other assessment scales may be used if necessary. The accompanying person is also trained in caring for the child during treatment. After completing the rehabilitation course, final testing is performed, and the patient's further care is determined. After treatment for the underlying condition is completed, the patient consults with a rehabilitation physician and receives recommendations. The patient has the opportunity to undergo rehabilitation at healthcare facilities at both the local and national levels.

Conclusion: In the Republic of Belarus, rehabilitation routing for patients with oncohematological diseases has been established, both during and after treatment. Proper patient routing helps improve the patient's quality of life.

Abstract 411

Cancer-Related Mortality in Children: A Retrospective Pathological Study

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Purpose: In Mongolia, the incidence of childhood cancer is 11.0 per 100,000 children, ranking 151st out of 185 countries worldwide, while mortality ranks 113th. Although immunohistochemistry and molecular pathology diagnostic technologies have been introduced into pediatric pathology services, improving diagnostic capacity, there remains an urgent need to investigate the underlying diseases and life-threatening complications associated with cancer-related mortality in children.

Methods: A retrospective case-based study was conducted on 85 cases of childhood cancer-related deaths examined at the National Center for Pathology between 2021 and 2025. Data sources included medical records, autopsy protocols, tissue blocks, and histological slides. Statistical analysis was performed using SPSS version 24.0.

Results: Among the 85 cases, age distribution was as follows: neonates 7 (8.2%), infants under 1 year 22 (25.8%), children under 5 years 17 (20.0%), and those under 18 years 39 (45.8%). Regarding hospital stay, 10 cases (11.7%) died within 24 hours, 17 (20.0%) within 1–3 days, 18 (21.1%) within 4–10 days, and 40 (47.0%) after more than 10 days of hospitalization. Hematologic malignancies accounted for 56 (65.8%), including 29 (51.7%) acute lymphoblastic leukemia, 25 (46.2%) acute myeloid leukemia, 2 (3.7%) B-cell lymphoma, and 1 (1.1%) Langerhans cell histiocytosis. Other malignancies included central nervous system tumors 11 (12.9%), neuroblastoma 3 (3.5%), osteosarcoma 3 (3.5%), hepatoblastoma 3 (3.5%), ovarian tumors 2 (2.3%), germ cell tumors 3 (3.5%), and benign tumors 3 (3.5%). The leading fatal complication was sepsis, observed in 46(54.1%). Among identified pathogens 15 (32.6%) were Staphylococcus,17 (36.9%) Streptococcus, 3 (6.5%) Escherichia coli, 5 (10.8%) Candida, 5 (10.8%) Aspergillus. Disseminated intravascular coagulation (DIC) was present in 21 cases (24.7%). Metastatic spread to organs such as the brain, heart, lungs, liver, kidneys, surrounding soft tissue, lymph nodes was identified in 66 cases (77.6%).

Conclusion: Hematologic malignancies are the leading cause of cancer-related mortality among children in Mongolia. The majority of life-threatening complications are associated with bacterial sepsis.

Abstract 412

Bridging the Survival Gap: Inequities in Childhood Cancer Outcomes Across Asia

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Purpose: Childhood cancer survival has improved dramatically in high-income countries (HICs), exceeding 80%, yet profound disparities persist globally. Asia bears nearly half of the global childhood cancer burden, but survival outcomes remain significantly lower and heterogeneous across the region. Understanding these inequities is critical to achieving the World Health Organization (WHO) Global Initiative for Childhood Cancer (GICC) target of 60% survival by 2030.

Methods: This narrative synthesis integrates evidence from global epidemiological reports, population-based cancer registries, and recent systematic and scoping reviews focusing on pediatric cancer outcomes in Asia and other low- and middle-income countries (LMICs). Key indicators analyzed include incidence, mortality, 5-year survival, and determinants of outcome disparities.

Results: Globally, over 400,000 children are diagnosed with cancer annually, with the majority of deaths occurring in LMICs. Survival exceeds 80% in HICs but remains below 30% in many LMICs, with Asia demonstrating a pooled 5-year survival of approximately 39.6%, far below Europe (74.3%) and North America (83.0%) . Within Asia, marked heterogeneity exists, with survival ranging from less than 10% in some countries to over 60% in high-income settings. Regional estimates indicate survival as low as 28.8% in parts of Southeast Asia compared to

over 50% in East Asia . Disease-specific outcomes also vary widely, with survival for key WHO index cancers ranging from 20% to over 90% across LMIC settings . These disparities are driven by multifactorial determinants, including delayed or missed diagnosis, limited access to essential medicines (available in only 29% of low-income countries), inadequate infrastructure, workforce shortages, treatment abandonment, and infection-related mortality . Additionally, fewer than 5% of children in Asia are covered by population-based cancer registries, highlighting critical data gaps that hinder effective policy and planning

Conclusion: Childhood cancer survival in Asia is characterized by profound inequities both between and within countries. Strengthening health systems, expanding access to timely diagnosis and standardized treatment, improving supportive care, and investing in robust cancer registries are essential to closing the survival gap. Regional collaborations through platforms such as SIOP Asia are pivotal in advancing equitable pediatric oncology care and accelerating progress toward global survival targets.

Abstract 413

Global and Regional Burden of Pediatric Malignancies Across Asia: Analysis from Global Burden of Disease 2023 Study

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Purpose: Pediatric malignancies represent a biologically and epidemiologically distinct group of cancers with substantial long-term impact. Although relatively rare, childhood cancers contribute disproportionately to global health loss. Asia, home to a large proportion of the world's pediatric population, faces unique challenges in addressing childhood cancer burden, particularly across low- and middle-income settings. Using estimates from the Global Burden of Disease Study 2023, this study evaluates the global and regional burden of pediatric malignancies with a specific focus on Asia.

Methods: We performed a secondary analysis of GBD 2023 cancer estimates, examining incidence, mortality, and disability-adjusted life years (DALYs) among children aged 0–14 years. Regional patterns were analyzed with emphasis on Asia, including disparities across income settings and cancer types. Key pediatric malignancies, including leukemias, central nervous system tumors, and embryonal tumors, were evaluated.

Results: Globally, pediatric cancers accounted for 1.4% of total cancer incidence and 1.0% of deaths, yet contributed disproportionately to 3.2% of total cancer DALYs, reflecting a high burden of premature mortality . Acute lymphoblastic leukemia and brain and central nervous system tumors were the leading contributors to childhood cancer burden. Asia bears a substantial share of this burden due to its large population base and demographic structure. Consistent with broader global trends, over 65% of cancer deaths occur in low- and middle-income countries, many of which are in Asia . Despite lower reported incidence, likely reflecting underdiagnosis, pediatric cancers in Asia are associated with disproportionately high mortality and DALYs, driven by delayed diagnosis, treatment abandonment, limited access to specialized care, and infection-related complications. GBD 2023 provides improved characterization of pediatric-specific malignancies including neuroblastoma, retinoblastoma, hepatoblastoma, and sarcomas highlighting their contribution to regional disease burden. The predominance of years of life lost (YLLs), accounting for the vast majority of cancer DALYs, underscores the critical impact of early mortality in children.

Conclusion: Pediatric malignancies in Asia represent a high-impact yet under-recognized public health challenge, marked by significant inequities in outcomes. Strengthening early diagnosis, expanding access to standardized treatment, improving supportive care, and enhancing cancer registry systems are essential to reducing disparities. Regional collaboration through platforms such as SIOP Asia will be pivotal in translating global evidence into context-specific strategies to achieve equitable childhood cancer outcomes across Asia.

Abstract 414

Clinical Characterization of Neurocutaneous Syndromes in Pediatric Oncology: A Single Centre Experience

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Purpose: Background: Neurocutaneous syndromes represent a heterogeneous group of genetic disorders with a predisposition to tumor development. Among these conditions, neurofibromatosis type 1 and tuberous sclerosis complex are the most encountered. Both disorders follow an autosomal dominant pattern of inheritance, with complete penetrance and variable expressivity. Aims: To describe the clinical features and assess pattern of family involvement in patients with neurocutaneous syndromes.

Methods: We retrospectively analysed the clinical data from pediatric patients presenting with clinical features suggestive of neurocutaneous syndromes at a Children's Cancer hospital Egypt 57357 between September 2024 and August 2025. Data collected included clinical presentation, radiologic findings, tumor characteristics and family history.

Results: Among 1,038 patients presenting with cancer during the study period, 65 (6%) fulfilled clinical criteria for a neurocutaneous syndrome. Fifty patients (5%) were diagnosed with neurofibromatosis type 1 (NF1), twelve (1%) with tuberous sclerosis (TS), and three with NF2-related schwannomatosis. Among patients with NF1, 26 (52%) developed optic pathway gliomas (OPG), nine had low-grade gliomas, five had plexiform neurofibromas, and one developed a malignant peripheral nerve sheath tumor. Thirteen patients with OPG (54%) presented with severe visual field impairment. Developmental delay was observed in 25 patients (50%), and 20 (40%) had a first-degree relative with NF1. Among patients with TS, eight developed subependymal giant cell astrocytomas (SEGA), five had renal angiomyolipomas, and three had cardiac rhabdomyomas. Developmental delay was observed in seven patients, and eight had a history of infantile spasms. Only one patient had a parent with TS. All three patients with NF2-related schwannomatosis had meningiomas; one had bilateral vestibular schwannomas, and two had a parent with a similar condition.

Conclusion: Structured surveillance programs and multidisciplinary monitoring are essential in neurocutaneous syndromes to facilitate early tumor detection, reduce complications, and improve long-term outcomes and quality of life for affected patients.

Abstract 415

Reduction of Chemotherapy Wastage in the Day Care Center of The Children's Hospital Lahore, Pakistan: Post-PrOFILE Quality Improvement Project

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Purpose: In resource-limited settings chemotherapy wastage results in a burden on the functioning of a pediatric oncology unit. We wanted to reduce chemotherapy wastage daily in the Day Care Center of The Children's Hospital Lahore that happens due to sick patient admissions in the ER and inpatient departments, as well as missed appointments caused by various socioeconomic factors and communication gaps. We aimed to reduce the chemotherapy wastage to less than 5% of daily chemotherapy prepared.

Methods: The current process of chemotherapy wastage was studied by using the IHI tools like Block diagram, Cause and Effect (Fishbone), and driver diagram. This study was conducted in the Daycare center of Children's Hospital from October 2024 to January 2025 as a basic course of the Quality Improvement Project in collaboration with IHI and St. Jude Global.

Results: During this study period, family engagement was promoted through training sessions regarding compliance with follow-up appointments. Staff engagement was promoted by keeping an accurate record of the logbook for wasted and reused chemotherapy. A hotline was established to inform about timely cancellation or rescheduling of appointments and sensitization of the whole PHO team with the strengthening of better communication among different stations of the unit. The analysis of process measures showed that the Hotline was used 60% of the time to communicate with patients' families, and 90%-time FN inpatient admissions were informed about cancellation for chemo. 60% of unused chemo utilized for other patients after dose adjustment, minimizing dose, drug, and storage-related mistakes.

Conclusion: By training, engaging, and promoting better communication among frontline workers, families, doctors, and nurses, these change ideas can overcome

the barriers of huge workload, physical separation of four stations of the PHO unit across different buildings and floors of the hospital to improve and could facilitate and help accomplish the chemo wastage reduction despite resource-limited settings in a low-middle income country like Pakistan.

Abstract 416

Comparison of methotrexate toxicity in pediatric cancer patients with and without therapeutic drug monitoring of serum methotrexate levels

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Purpose: Methotrexate (MTX) is a key chemotherapeutic agent widely used in the treatment of pediatric malignancies; however, it can cause a range of toxic effects, including myelosuppression, mucositis, hepatotoxicity, nephrotoxicity, and neurotoxicity. In Mongolia, limited availability of laboratory diagnostics for measuring serum MTX levels previously restricted the implementation of therapeutic drug monitoring (TDM). Consequently, clinicians often reduced the standard therapeutic dose in routine practice to minimize the risk of severe toxicity. Since 2025, laboratory testing for serum MTX levels has been introduced into clinical use through the project “Global Platform for Improving Access to Medicines for Childhood Cancer,” enabling TDM to guide safer chemotherapy administration. This study aimed to compare the severity and frequency of MTX-related toxicities in pediatric cancer patients receiving chemotherapy with and without monitoring of blood MTX levels.

Methods: This retrospective study was conducted at the Center for Pediatric Hematology and Oncology of the National Center for Maternal and Child Health (NCMCH). Pediatric patients who were hospitalized and treated with MTX were included. Adverse events and treatment-related toxicities were assessed and graded according to the National Cancer Institute Common Terminology Criteria for Adverse Events (CTCAE), version 3.0, with toxicity severity classified from grade 0 to grade 4. Hematologic, hepatic, renal, and neurologic toxicities associated with MTX therapy were evaluated and compared between patients with and without MTX level monitoring.

Results: A total of 30 methotrexate treatment episodes were analyzed. Females accounted for 53.3% of cases, and the median age was 9.5 years (IQR 5.3–14.0). Methotrexate serum levels were measured in 23.3% of cases. The most frequent diagnosis was C91.0 (60.0%), and the median methotrexate dose was 4,996 mg (IQR 2,886.3–5,576.0). Methotrexate concentrations decreased from 49.7 ± 24.8 at 24 hours to 5.72 ± 10.3 at 48 hours and 1.49 ± 2.22 at 72 hours ($p < 0.001$). Anemia and hepatotoxicity occurred in all patients. Leukopenia, neutropenia, thrombocytopenia, and nephrotoxicity occurred in 70.0%, 50.0%, 36.7%, and 16.7% of cases, respectively. Grade 4 hepatotoxicity was the most common severe toxicity (86.7%). Leukopenia was significantly more frequent in patients without methotrexate level monitoring ($p = 0.014$).

Conclusion: MTX-related toxicities remain common among pediatric oncology patients receiving chemotherapy, particularly hepatotoxicity and hematologic toxicity. The significantly higher frequency of leukopenia in patients without MTX monitoring suggests that therapeutic drug monitoring may enhance treatment safety and support optimized MTX dosing in pediatric cancer care.

Abstract 417

THE STATUS OF CHILDREN'S CANCER IN MONGOLIA AND COMPARISON WITH WORLD DATA

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Purpose: Almost 90% of children with cancer live in low- and middle-income countries, where only about 20% survive the disease. This highlights the critical importance of expanding research on childhood cancer in these settings. Objective: To describe the epidemiological patterns of childhood cancer in Mongolia and explore associated factors.

Methods: A narrative mini-review was conducted using peer-reviewed articles published between 2000 and 2026 that addressed childhood cancer epidemiology and risk factors.

Results: Globally, an estimated 400,000 children are newly diagnosed with cancer each year. In Mongolia, 148 new cases were reported among children aged 0–19 in 2024. Childhood cancer incidence is closely linked to economic development, industrialization, and environmental exposures. Evidence indicates that residential pesticide exposure, electromagnetic fields, parental smoking and alcohol use, as well as birth weight, significantly increase the risk of childhood leukemia.

Conclusion: In Mongolia, childhood cancer diagnosis and treatment are influenced by socioeconomic conditions. There is one main hospital with a devoted unit of a few oncologists taking on the childhood cancer battle in Mongolia, treating approximately 100 new patients per year. Mongolia has successfully initiated a collaboration through a joint program between St. Jude Children's Research Hospital and the World Health Organization (WHO) to gain access to Childhood Cancer Medicines. This is one of the great achievements in reducing the economic burden on patients’ households in Mongolia. Through this program, patients with cancer can acquire high-quality, original medicines at no cost. Ultimately, increasing awareness of modifiable risk factors is crucial for prevention, and the epidemiological pattern of childhood cancer in Mongolia aligns with global trends.

Abstract 418

Time to Antibiotic Administration in Pediatric Oncology Patients Presenting to the Emergency Department with Fever and Neutropenia

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Purpose: Background: Pediatric oncology patients receiving chemotherapy are at increased risk of severe infections due to impairment of both innate and adaptive immunity. Fever may be the only presenting sign of infection because classical inflammatory responses are often blunted. Delay in antibiotic administration can lead to rapid progression of infection and sepsis. Early antimicrobial therapy has therefore become a key goal in the management of febrile neutropenia. Previous studies have shown that administration of antibiotics within the first 60 minutes of presentation is associated with improved outcomes. Aims: 1. To evaluate the time to antibiotic administration (TTA) in pediatric oncology patients presenting with febrile neutropenia. 2. To assess patient outcomes associated with delayed antibiotic administration (>60 minutes). 3. To identify barriers contributing to delays between triage and antibiotic administration in the emergency department.

Methods: A descriptive cross-sectional cohort study was conducted at The Indus Hospital and Health Network, Karachi, Pakistan, from April 2021 to September 2021. A total of 357 patients were included using the WHO sample size calculator with a significance level of 0.05. Data collected included oncologic diagnosis, absolute neutrophil count (ANC), triage time, time to antibiotic order, barriers to timely antibiotic administration, and patient outcomes within 24 hours. Data were analyzed using SPSS version 24. Chi-square test was applied to determine associations, with $p < 0.05$ considered statistically significant

Results: Among 357 patients, the mean age was 7.40 ± 4.13 years. Males constituted 66.7% of the sample. Severe neutropenia was observed in 41.2% of patients. The mean triage time was 8.26 ± 3.29 minutes, and the mean TTA was 61.93 ± 25.55 minutes. Antibiotics were administered within 60 minutes in 71.4% of cases, while 28.6% experienced delays. Common barriers included patient overload (24.5%), staff-related delays (85.3%), doctor-related delays (93.1%), and patient-related factors (72.5%). Patient outcomes included discharge (9.0%), daycare follow-up (48.7%), ward admission (29.4%), HDU admission (8.7%), ICU admission (4.2%), and mortality (2.2%). Outcomes were not significantly associated with TTA ($p > 0.05$), although ICU admission was significantly higher among patients receiving antibiotics within 60 minutes ($p = 0.012$).

Conclusion: Delay in antibiotic administration beyond 60 minutes was not significantly associated with adverse outcomes in this cohort. While prompt antibiotic administration remains important, clinical judgment and individual patient assessment should guide management decisions.

10. NURSING — ORAL PRESENTATION

Abstract 419

A Short Art Therapy Program to Reduce Stress and Burnout Risk Among Nurses in Armenia

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Purpose: Nursing is associated with a high physical and emotional workload, shift-based schedules, and continuous responsibility, which can contribute to cumulative stress and the development of occupational burnout. Burnout may manifest as emotional exhaustion, detachment, and reduced professional motivation. Feasible, low-cost self-care interventions delivered in the workplace may support nurses' well-being and team stability. Objective: To assess the impact of an 8-week art therapy program in Armenia on nurses' perceived stress and burnout indicators, and to provide a safe space for self-expression and reflection on personal challenges.

Methods: A pilot pre–post study will be conducted with 20 nurses. The intervention will consist of one weekly group session (1–1.5 hours) over 8 weeks, including structured activities such as coloring, drawing, collage-making, and expressive writing in a safe, non-judgmental environment. Outcomes will be assessed before and after the program using validated stress and burnout questionnaires, alongside participation and satisfaction measures.

Results: We anticipate reduced perceived stress and emotional exhaustion, improved mood and work engagement, and strengthened perceived team support.

Conclusion: A short workplace-based art therapy program may be a feasible and beneficial approach to enhance nurses' well-being and reduce burnout risk in Armenia by promoting self-expression and psychological recovery.

Abstract 420

Implementing a PEDS-DTRS–Based Psychosocial Distress Algorithm for Children with Cancer in an Indonesian Tertiary Hospital

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Purpose: Children diagnosed with cancer not only have physical symptoms but also psychological ones which are often not recognized. Limited screening tools are one of the contributing factors, causing delays in intervention for psychosocial problems. With the Pediatric Distress Thermometer Rating Scale (Peds-DTRS) developed by Patel et al as a screening tool and intervention algorithm for psychosocial problems prepared based on literature, it is hoped that the level of distress and psychosocial issues that occur in children with cancer can be detected.

Methods: This is a Quality Improvement Project (QIP) that uses the PDCA method (plan, do, check, action) with 30 pediatric patient participants aged 7 to 18 years.

Results: The average distress scale was 2.53, with a low distress level of 46.7%, moderate distress (26,7%), no distress (23,3%), and 3,3% high distress. Almost all participants experienced emotional (93.3%) and physical problems (96.6%). Based on the algorithm, in addition to education and standard interventions, 20% of participants were referred to psychologists and 20% to child psychiatry, 13.3% received psychopharmaceuticals, and 6.7% were referred to the palliative team.

Conclusion: The problem intervention algorithm can detect psychosocial distress and assist health workers in determining interventions or referrals based on the problems experienced by pediatric patients.

Abstract 421

Understanding nurses' perspectives on palliative care by assessing their knowledge, attitudes and experiences

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Purpose: Knowledge in palliative care (PC) is crucial in providing holistic, compassionate and patient-centered care to patients facing life-limiting illnesses. As nurses are at the forefront of patient care, a strong understanding of PC principles is vital in addressing the unique physical, psychological, social and spiritual needs of patients. Aim: Explore baseline knowledge, attitudes, and experiences regarding PC among nurses.

Methods: A cross-sectional study was conducted among 500 nurses employed at KK Women's and Children's Hospital, Singapore, between February and May 2025. Demographic variables including age, years of clinical experience and prior PC training were collected. Knowledge of PC was assessed using the Palliative Care Knowledge Test (PCKT), while attitudes towards end-of-life care were evaluated using Frommelt's Attitude Toward Care of the Dying Scale (FATCOD-B). Self-reported PC practices were assessed using the Palliative Care Self-Reported Practices Scale (PCPS). Data were analyzed using descriptive statistics and between-group differences were examined using Kruskal-Wallis test.

Results: Of the 500 nurses invited, 332 (66.4%) completed the questionnaire. Mean scores indicated moderate PC knowledge (PCKT: 10.96 ± 3.69 /20), practice confidence (PCPS: 59.59 ± 12.87 /90), and attitudes towards end-of-life are (FATCOD-B: 100.92 ± 8.77 /150). Prior PC education and clinical experience were consistently associated with higher knowledge and practice confidence, with significant effects observed in dypsnoea domain of PCKT ($H(1)=4.627$, $p=0.031$) and across all PCPS domains ($H(1)=6.754$, $p=0.009$), as well as overall PCKT ($H(1)=4.283$, $p=0.038$) and PCPS scores ($H(1)=7.615$, $p=0.006$). Higher nursing qualifications were also associated with greater practice confidence ($H(4)=12.097$, $p=0.017$). In contrast, PC outcomes did not differ significantly by religion, gender or years of clinical experience.

Conclusion: The results underscore the importance of integrating structured PC educational and clinical program to address existing gaps in knowledge, promote more favourable attitudes towards the provision of PC and strengthen PC delivery in the current setting.

Abstract 422

CAREGIVER MEDICATION INFORMATION NEEDS AND SERVICE FACTORS ACROSS HOSPITAL-HOME TRANSITIONS IN PAEDIATRIC ONCOLOGY

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Purpose: As paediatric oncology shifts toward ambulatory care, caregivers assume responsibility for complex, high-risk medication regimens across fragmented service settings. This study aimed to identify and describe caregivers' medication information needs and preferences and to explore service- and system-level factors that support or impede those needs being met across hospital-home transitions.

Methods: A qualitative descriptive study was conducted at a tertiary paediatric oncology centre in Australia. Semi-structured interviews were undertaken with 15 caregivers of children receiving active treatment or in maintenance/remission; 60% of caregivers were born overseas. Participants were purposively sampled across diagnoses and treatment phases. Interviews were analysed using reflexive thematic analysis.

Results: Caregivers described medication information needs as evolving across treatment phases and intensifying at care transitions. Ten caregivers reported discrepancies between verbal advice, written materials, and electronic records during discharge or inpatient-outpatient shifts. Caregivers routinely reconciled conflicting instructions, verified dosages, and monitored inpatient administration, positioning themselves as informal safety checkpoints across institutional boundaries. Nine caregivers managing children in active treatment described sustained polypharmacy, night-time dosing, formulation manipulation, and frequent dose clarification. In contrast, caregivers in maintenance reported episodic information needs linked to isolated regimen changes. Demonstration-based and multimodal education enhanced usability, particularly for dose measurement and handling of hazardous medications. Several overseas-born caregivers described uncertainty when interpreter access was limited, relying on family translation or digital tools to clarify instructions. To maintain continuity, caregivers developed portable medication summaries, written logs, alarm systems, and stock-monitoring routines to compensate for communication gaps across settings.

Conclusion: Caregivers' medication information needs in paediatric oncology are shaped by service design and transition processes rather than knowledge gaps alone. Structured, staged, and linguistically accessibly education embedded within

discharge and outpatient pathways may strengthen caregiver preparedness and medication safety across hospital-home boundaries.

Abstract 423

Economic Toxicity and Quality of Life Among Caregivers of Children with Cancer: The Mediating Role of Decision-Making Self-Efficacy

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Purpose: To examine the impact of economic toxicity on quality of life among caregivers of children with cancer and verify the mediating role of decision-making self-efficacy in this relationship.

Methods: A cross-sectional study was conducted on 369 caregivers from a tertiary pediatric oncology center with a mean age of 38.2±6.3 years and 69.1% being mothers. The Cancer Family Caregiver Economic Toxicity Scale, 10-item Connor-Davidson Resilience Scale (CD-RISC-10), Decision Self-Efficacy Scale and Pediatric Quality of Life Inventory (PedsQL™) Family Impact Module (FIM) 2.0 were used for investigation. Parallel mediation analysis with 5000 bootstrap resamples was performed using Mplus 8.11.

Results: Economic toxicity was negatively correlated with decision-making self-efficacy (r=-0.131, P=0.012) and quality of life (r=-0.456, P<0.001), while decision-making self-efficacy was positively correlated with quality of life (r=0.282, P<0.001). Economic toxicity had a significant direct negative effect on quality of life (β=-0.718, P<0.001), and decision-making self-efficacy exerted a significant mediating effect between them with an indirect effect of -0.042 (95%CI: -0.089~-0.003, P<0.05), accounting for 7.1% of the total effect.

Conclusion: Economic toxicity directly impairs the quality of life of caregivers of children with cancer and this effect is partially mediated by reduced decision-making self-efficacy. It is recommended to integrate economic burden screening and decision-making self-efficacy training into clinical supportive care for this population to alleviate the adverse impact of economic toxicity.

Abstract 424

REDUCESES THE PHLEBITIS AMONG PEDIATRIC ONCOLOGY POPULATION AFTER PROVIDED CARE AT TIME OF INSERTION, MANAITANCE AND REMOVAL

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Purpose: Peripheral venous cannulation is a common procedure used in hospital to deliver fluid & medicine. Phlebitis (inflammation of the vein can be caused by chemical, mechanical or infectious irritation. Thrombophlebitis is due to one or more blood clots in a vein that cause inflammation. Thrombophlebitis in neutropenic patients is a serious, often septic, condition characterized by vein inflammation and blood clots, commonly associated with cancer treatment. It frequently results from catheter-related infections, presenting with fever, pain, and redness Aim and objective: To reduce the frequency of phlebitis and infection rates in pediatric oncology patients.

Methods: The observational prospective study investigated to observe the phlebitis and blood stream infection rates among paediatric population from October 2025 to January 2026 by the Infection control department of children hospital to compared the rate of infection and phlebitis before and after intervention of patients between 1-10 years of age. Interventional activities including staff education and demonstration and continuous observation applied care bundle

Results: Before intervention we have observed 507 patients have peripheral line and 16 (3.1%) have developed phlebitis grad 2 and 3, after intervention 394 patients were observed 0% phlebitis and blood stream infection.

Conclusion: Studies in various setting been showed the result that infections related to devices can be reduced by adopting aseptic techniques, choosing the catheter bore and puncture site appropriately, and using the best scientific evidence, could contribute to success in reducing phlebitis related to the use of peripheral intravenous devices. The implantation of institutional protocols and care guidelines, which aim to prevent phlebitis, are essential for safe care. Considering that phlebitis is an adverse event which it is possible to prevent, nurses have a central role in care involving intravenous therapy.

11. NURSING — POSTER

Abstract 425

It’s My Life: An Ethical Analysis of Parent-Adolescent Decision-Making Conflict Over Cancer Treatment

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Purpose: One of the most challenging situations for health care providers arises when a teenager refuses treatments that their parents or legal guardians insist on, believing or having been told by the clinician, that these treatments are lifesaving or life-prolonging. Although in most countries, parents generally have legal authority for persons less than 18 years old, it is widely recognized from an ethical standpoint that adolescents should play an active role in decision-making. Background: Adolescent refusal of treatment is an ethically challenging issue, especially when parents and clinicians support continued life-saving care. Although legal authority usually rests with parents, adolescents should still be involved in decision-making. This is particularly important in oncology, where treatment burden, uncertainty, and quality-of-life concerns are significant. Aim: To explore the ethical aspects of adolescent treatment refusal in pediatric oncology and highlight the role of nurses and interdisciplinary collaboration in supporting decision-making

Methods: The 4-topic method uses 4 foci of inquiry (medical indications, patient preferences, quality of life, and contextual features) to facilitate the incorporation of all aspects of a situation. These topics “help clinicians understand how the ethical principles connect with the circumstances of the clinical case” and the patient’s particular contextual circumstances. Furthermore, the topics facilitate consideration of different aspects of the problem and serve as focal points for data gathering. Questions under each topic serve as prompts for inquiry relevant to the situation. Case exploration is iterative, moving back and forth among the topics as the story unfolds and new information is discovered. Here we use M.L.’s case to demonstrate how information gathering proceeds using the 4 topics as a guide.

Results: After his symptoms were better managed and he had an additional discussion with the psychiatrist, M.L. became more open to further conversation. A team meeting, attended by Anya at M.L.’s request, helped both M.L. and his parents recognize that they shared the same goal: improving his comfort and well-being. The discussion revealed M.L.’s previous sense of isolation and lack of trust in being heard. Following a review of expected outcomes with and without treatment, M.L. agreed to a trial of outpatient chemotherapy with palliative care support for symptom management, along with counseling for emotional distress. A shared timeline for reassessment was also established. The case highlights the importance of giving adolescents as much involvement and control as reasonably possible, even when legal decision-making authority remains with parents.

Conclusion: This case shows that adolescent treatment refusal is not simply a conflict, but often reflects different views of what is best for the patient. Ethical analysis and open communication can help families and care teams find common ground. Nurses play a key role in advocacy, trust-building, and interdisciplinary collaboration, making their involvement essential in resolving complex decisions in pediatric oncology.

12. OTHER — ORAL PRESENTATION

Abstract 426

The Double Burden of War: Physical and Psychological Impact of the 2024 Lebanon Conflict on Children with Cancer and Blood Disorders

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Purpose: The recent Middle East war extended its reach to Lebanon in 2024, where conflict and widespread displacement affected treatment plans in pediatric patients suffering from cancer and blood disorders. The study's main objectives were to quantify the prevalence of displacement and medical care barriers among pediatric oncology patients during the war, to assess any symptoms deterioration from pre-war to wartime, and to explore unmet needs for professional psychological support conflict-affected patients and their caregivers.

Methods: Parents of 60 pediatric patients aged 1–18 with cancer or blood disorders were identified through clinical records, and were invited to participate in an anonymous online survey assessing the physical and psychological changes their child experienced during the war relative to the baseline, resulting in a 50% response rate. Descriptive analysis was used, and ethical approval was obtained from the Lebanese American University IRB.

Results: 63.3% of participants reported being displaced during the war. Around 33.3% of pediatric patients were unable to obtain necessary medications, and nearly 50% were unable to visit a doctor or hospital at times during the war. Financial difficulties and unsafe commute conditions were the most reported barriers. 50% of children experienced deteriorated physical health, with fatigue and muscle pain being the major symptoms reported. Psychologically, 76.7% were markedly emotionally distressed, and 83.3% felt increasingly scared during the war. One-third of children entirely stopped learning, and 83.3% of patients reported they would benefit from professional psychological help.

Conclusion: War and displacement in Lebanon have exacerbated both physical and psychological struggles faced by children with cancer and blood disorders. This study identifies gaps in psychological support and calls on governments, hospitals, and local and international organizations to develop more effective and comprehensive care plans and establish better support systems for children with cancer in conflict settings.

Abstract 427

Patterns of Pain and Pain Management in Pediatric Oncology and Hematology Patients: Experience from a Tertiary Care Center in Pakistan

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Purpose: Pain is a major source of distress in children with cancer, yet pediatric palliative care (PPC) services in low- and middle-income countries remain limited. In Pakistan, inadequate physician training, misconceptions, and restricted opioid use contribute to poor pain management. This study aimed to assess the prevalence, characteristics, and approach to pain management in pediatric hematology-oncology, along with self-reported physician competency at a hospital in Pakistan where PPC services were initiated.

Methods: A prospective descriptive study was conducted from January–June2024 at The Children's Hospital, Pediatric Hematology-Oncology, department, Lahore. Children (0–18 years) with moderate to severe pain were enrolled. Pain was assessed using age-appropriate validated scales, while adequacy of pain management and physician knowledge were evaluated through chart reviews and structured surveys.

Results: Among 724 admitted patients, 390 (54%) reported pain. Of these, 78 (20%) patients had severe pain, while 140 (36%) and 172 (44%) patients reported moderate and mild pain, respectively. Eighty patients with moderate to severe pain were enrolled. The median age was 6.5 years, with a male predominance (61%). The most common sites of pain included headache (21%), generalized body aches (12.5%), and abdominal pain (10%). Paracetamol was the most frequently prescribed analgesic, followed by tramadol and morphine. Intravenous routes were preferred, and combination therapy was common. Tramadol was predominantly administered intravenously, and most recipients (86%) were under 12 years of age. Morphine use was variable, with low oral use (11%); 31% received IV morphine regularly and 30% as needed, while 39% of patients with moderate to severe pain received no strong opioids. Overall adherence to WHO pain management guidelines was low (12%), and physician surveys indicated limited familiarity with validated pain scales, opioid prescribing, standard protocols, and non-pharmacological pain management techniques

Conclusion: Pain is highly prevalent in pediatric oncology patients but remains inadequately managed due to poor management practices. Strengthening PPC services through standardized protocols, education, and improved access to essential analgesics is urgently needed to enhance the quality of life for children with cancer and their families.

Abstract 428

SURVIVORS' PROFILING EXERCISE (SUPERSG) : DEVELOPING A BASELINE UNDERSTANDING OF QUALITY OF LIFE OF CHILDHOOD CANCER SURVIVORS' IN SINGAPORE

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Purpose: Childhood cancer survivors (CCS) quality of life (QOL) outcomes in Southeast Asia remains underrepresented in the quality-of-life literature. Specifically, in Singapore, no population-level quality of life study has been conducted to determine the QOL of CCS. This study aimed to establish a national QOL profile of childhood cancer survivors, to aid the development of better and more appropriate support for survivors.

Methods: A cross-sectional study was conducted with 254 survivors diagnosed between 2007 and 2023 who had completed treatment. The sample represented approximately 21% of the Singaporean CCS population. Age-appropriate validated instrument was used, including Pediatric Quality of Life Inventory (PedsQL), KidSCREEN and World Health Organization Quality of Life (WHOQOL). Scores were compared with published normative Singapore population data and subgroup analysis was conducted to identify particular groups of survivors with poorer quality of life.

Results: Overall QoL scores were comparable to population norms across most domains, suggesting most survivors are doing comparable to their healthy counterparts. However, psychological wellbeing remains consistently lower across all age groups. We also found that survivors who recently completed treatment who reported lower physical well-being while long-term survivors indicated lower social well-being. Although 91% of the survivors were receiving follow-up care, only 45% reported experiencing or suspecting late effects, suggesting potential gap in late effect recognition.

Conclusion: This study provides one of the first national-level survivorship QOL baseline in Singapore. Its findings indicated the need to update our approach towards long-term survivorship care: it underscores the need for sustain psychological support, introducing physical activity earlier in survivorship, the need for long-term social reintegration strategies and a strengthened survivorship education. Lastly, the multi-instrument age-appropriate design offers a scalable framework for survivorship QoL monitoring in other contexts.

Abstract 429

Common respiratory virus detection by targeted next-generation sequencing in pediatric hematological patients: a stratified analysis by HSCT status and underlying disease

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Purpose: Pediatric patients with hematological diseases are at high risk for respiratory viral infections due to immunosuppression. Targeted next-generation sequencing (tNGS) enables comprehensive virus detection, but data on virus epidemiology across different clinical contexts in Asian pediatric populations remain limited.

Methods: We retrospectively analyzed 970 tNGS samples from 579 pediatric hematological patients including both malignant and benign conditions with suspected respiratory infections at the Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences, Tianjin, China. Samples were stratified by hematopoietic stem cell transplantation (HSCT) status at sampling (HSCT: 136 patients, 230 samples; non-HSCT: 497 patients, 740 samples) and further by underlying disease type (benign vs. malignant). Detection rates of nine common respiratory viruses and viral co-infection patterns were compared between groups.

Results: Overall virus detection was comparable between HSCT and non-HSCT samples (38.7% vs. 34.6%, P=0.284). However, malignant disease samples had significantly higher detection than benign samples (37.4% vs. 25.3%, P=0.004). Detection rates varied across four subgroups: HSCT-malignant (41.7%), non-HSCT-malignant (36.3%), HSCT-benign (29.9%), and non-HSCT-benign (21.8%). Viral co-infections (≥2 viruses) were more frequent in HSCT samples (21.3% vs. 10.9%, P=0.011). Adenovirus (4.3% vs. 1.1%, P=0.001) and bocavirus (2.6% vs. 0.5%, P=0.009) were more common in HSCT samples. Within the HSCT group, rhinovirus predominated in malignant samples (16.6% vs. 4.5%, P=0.013). Seasonally, influenza virus and RSV showed typical winter peaks, while rhinovirus peaked in autumn (P<0.001).

Conclusion: Malignant disease, rather than HSCT status alone, is the dominant risk factor for respiratory virus detection in pediatric hematology patients. Higher viral co-infection rates and adenovirus/bocavirus detection in HSCT patients suggest virus-specific vulnerabilities. These findings inform risk-adapted infection control strategies in Asian pediatric hematology settings.

Abstract 430

Late Effects and Social Adaptation of Neuroblastoma Patients Following Therapy Completion: Results of a Cross-sectional Parental Survey

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Purpose: Neuroblastoma (NB) patients can suffer different late side effects, that can increase the risk of neurobehavioral disorders and lead to impaired social adaptation. The aim was to evaluate late effects and social adaptation in NB survivors

Methods: A questionnaire was administered to NB survivor families on site during follow-up visits with items including primary disease, treatment, social activity, psychological difficulties and the presence of chronic conditions. Patients were diagnosed from 01/2012 to 12/2019 and treated according to a modified German NB-2004 protocol at Dmitry-Rogachev National-Medical-Research-Center of Pediatric Hematology, Oncology and Immunology. The questionnaire for parents included kindergarten/school attendance, teaching format, school performance, memory problems, decreased activity, concentration problems, educational difficulties, additional psychological support and more.

Results: 459 families were included in the analysis (low-risk:368, intermediate-risk:40, high-risk:51 patients).Median age at diagnosis and survey was 9,5 months (range: 0.06 – 205.4) and 100 months (range: 50.5–307.6). Late effects were observed in 261/459 patients (57%), with a median of 2 late effects per patient (range: 1–7). The most frequent late effects were: musculoskeletal disorders(38%), respiratory disorders(24%), gastrointestinal disorders(23%), renal(19%), endocrine(11%), neurological(8%), visual disorders(7%), cardiovascular disorders(4%), hearing loss(8,7%) and second malignancies(2%). Neurobehavioral impairment was reported in 135 patients (29%). Social adaptation difficulties were observed in 22% of low-risk patients (81/368) compared with 59% in patients of intermediate/high-risk groups (54/91) (p<0.001; OR = 5.17). Patients with more than two late effects (n=103) and those of intermediate/high-risk groups (n=91) more often had memory problems, reduced activity, attention difficulties, learning problems, communication difficulties with peers, and need for psychological support (all p<0.001).

Conclusion: NB survivors are at high-risk for late effects that can impair functionality and social adaptation. Parents should be informed, patients should be followed accordingly and the interdisciplinary treatment team should support the families from diagnosis to late follow-up

13. OTHER — POSTER

Abstract 431

Strengthening early childhood cancer detection: A qualitative study of awareness training

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Purpose: Delayed diagnosis and limited awareness remain major barriers to improving childhood cancer outcomes in Bangladesh. To address these challenges, World Child Cancer, supported by Bristol Myers Squibb, implemented Early Warning Signs and Symptoms (EWSS) training across six major medical colleges and hospitals in Bangladesh aimed to strengthen healthcare professionals' ability to detect early signs of childhood cancer and facilitate timely referral for specialised treatment. This study examined the outcomes of this focused training intervention.

Methods: Eight one-day training sessions were conducted across six major medical colleges and hospitals in Bangladesh. Led by senior paediatric oncology specialists, the sessions followed an interactive format combining lectures, case-based discussions and Q&A. Core content covered early detection, tumour-specific symptom recognition, available treatment pathways, and the importance of early referral. A qualitative assessment guided by Organisation for Economic Co-operation and Development evaluation criteria was conducted to assess the training's effectiveness. Semi-structured interviews were conducted with a representative sample of participants, focusing on training methods, learning outcomes and application of knowledge gained in practice.

Results: The training effectively addressed key gaps in early childhood cancer detection across clinical roles and successfully trained 483 doctors and medical students across six medical facilities. Participants reported the application of EWSS knowledge to enhance early detection, and improved ability to recognise signs such as unexplained abdominal discomfort or bleeding as possible malignancies. Respondents agreed that early identification has the potential to improve childhood cancer survival, reduce treatment time and costs, and enable timely intervention.

Conclusion: EWSS training strengthened healthcare professionals' ability to recognise early cancer symptoms and improved their readiness to act on potential warning signs. The training demonstrated clear qualitative impact while highlighting opportunities to strengthen training duration and reinforcement materials to improve long-term effectiveness.

Abstract 432

Kasabach–Merritt Phenomenon In A Giant Facial Hemangioma With Chronic Coagulopathy And Iron Deficiency Anemia

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Purpose: Kasabach–Merritt phenomenon (KMP) is a life-threatening consumptive coagulopathy from large vascular tumors, featuring platelet trapping, coagulation activation, and anemia. Chronic intralesional bleeding can cause iron deficiency. We report an adult with giant facial hemangioma, KMP, chronic coagulopathy, and iron deficiency anemia managed multimodally.

Methods: A 39-year-old male with hematologic surveillance presented with recurrent gingival bleeding and facial asymmetry from a giant hemangioma. Labs showed hemoglobin 118 g/L, RBC 5.42 × 10^{12}/L, MCV 67.7 fL, MCH 21.8 pg, platelets 358 × 10^{9}/L, ferritin 21.8 ng/mL (microcytic iron deficiency anemia). Coagulation: aPTT 37.9 s, PT 13.5 s, INR 0.99, fibrinogen 4.06 g/L, TT 15.8 s. Prior treatments: embolization, radiation, partial resection. Support: FFP, IV iron. Current: sirolimus and CT angiography every 6 months.

Results: The lesion caused persistent hemostatic imbalance with localized intravascular consumption. Despite normal platelets, history confirmed tumor-related sequestration, coagulation activation, and chronic bleeding-induced iron deficiency. Sirolimus stabilized lesion size and bleeding under multidisciplinary care.

Conclusion: Adult KMP is rare and challenging. Giant vascular tumors can drive localized coagulation, platelet consumption, and iron deficiency without profound thrombocytopenia. Early antiangiogenic therapy (e.g., sirolimus), iron correction,

and serial imaging prevent hemorrhagic/thrombotic risks. Multidisciplinary management ensures long-term hemostatic stability.

Abstract 433

From Scarcity to System: Strengthening Pediatric Palliative Care in Pakistan Using the WHO Public Health Mod

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Purpose: Pediatric palliative care (PPC) is an essential component of comprehensive child health services, and is particularly important in countries with a high burden of life-limiting illnesses. In Pakistan, where over 60% of the population is under 18 years, children experience significant suffering from cancer, genetic, neurological, and metabolic disorders. Despite growing worldwide recognition , PPC remains underdeveloped. The World Health Organization (WHO) Public Health "House Model" provides a structured framework to assess palliative care systems across six domains. We reviewed Pakistani PPC provision against performance indicators from the WHO Model to identify context-specific adaptations that better address the needs of Pakistani children and families.

Methods: We conducted a contextual analysis of pediatric palliative care in Pakistan using the World Health Organization Public Health Model for Palliative Care as a conceptual framework. The six domains of the model empowered communities, health policy, research, essential medicines, education and training, and service provision were used to examine current system challenges and areas requiring strengthening. Insights from informal discussions with clinicians were considered alongside existing knowledge of the health system to highlight key challenges across the six domains of the WHO framework.

Results: We identified key gaps across all domains. Public awareness of PPC is limited, with cultural stigma surrounding opioids and serious childhood illness. National policies are largely adult-focused, with no formal integration of PPC into child health frameworks or referral pathways. Research output and national registries are minimal. Access to essential medicines, particularly oral morphine and pediatric formulations, is inconsistent due to regulatory barriers and limited training in opioid use. Undergraduate and postgraduate PPC education is scarce. Service provision is confined mainly to tertiary centers, with late referrals and limited home-based care models.

Conclusion: Strengthening PPC in Pakistan will require integration into national child health policies, development of pediatric-specific guidelines, improved access to essential medicines, structured training programs, expanded service delivery models, and promotion of locally relevant research. Coordinated policy action and investment are essential to ensure early, equitable, and high-quality palliative care for every child in need.

Abstract 434

MAINTAINING ACCESS TO UNRELATED ALLO-HSCT UNDER EXTERNAL CONSTRAINTS: EXPERIENCE FROM BELARUS

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Purpose: Allogeneic hematopoietic stem cell transplantation (allo-HSCT) from unrelated donors expands treatment options when no matched sibling donor is available. Access to international registries enables curative therapy for malignant hematologic diseases, immunodeficiencies, and inherited metabolic disorders. However, donor procurement and cell delivery are highly vulnerable to external disruptions affecting communication, transportation, and financial operations.

Methods: Allogeneic HSCT in Belarus is performed at two national centers: an adult transplant unit with 27 beds and a pediatric center with 8 beds for children, adolescents, and selected young adults. National data on unrelated allo-HSCT performed between 2018 and 2025 were analyzed, with particular attention to external factors influencing donor procurement and logistics.

Results: A total of 445 allogeneic HSCT procedures were performed during the study period, including 275 (61.8%) from unrelated donors. Annual numbers varied substantially. Procedures increased from 30 in 2018 to 32 in 2019, dropped to 16 in 2020 during the COVID-19 pandemic, recovered to 32 in 2021, declined again to 27 in 2022 amid geopolitical disruptions, and then rose sharply to 47 in 2024 and 67 in 2025. The national Central Registry identified 42 donors (15.3%) domestically, while 233 donors (84.7%) were sourced through international registries. The pandemic disrupted transport networks, requiring specialized courier services and government support for cellular product delivery. From 2022 onward, additional challenges included financial transaction barriers, transit restrictions, and reliance on third-country routes and land transport. Force majeure events often necessitated an urgent route modification within a 72-hour permissible

Conclusion: Despite major external disruptions, Belarus preserved and ultimately expanded access to unrelated allo-HSCT. Although procedure numbers temporarily declined, the subsequent increase demonstrates the effectiveness of adaptive logistical strategies and cross-sector collaboration. Continued optimization of communication channels, transportation pathways, and financial mechanisms remains essential to ensure reliable delivery of cellular products and uninterrupted access to life-saving transplantation.

Abstract 435

Advancing Early Referral Pathways for Childhood Cancer: Insights from Early Warning Signs and Symptoms Training in Bangladesh

Dipika Joshi
World Child Cancer

Purpose: Delayed diagnosis and limited awareness of early cancer symptoms remain major barriers to improving childhood cancer outcomes in Bangladesh. To address these challenges, World Child Cancer, supported by Bristol Myers Squibb, implemented Early Warning Signs and Symptoms (EWSS) training across six major hospitals in Bangladesh. This study explored the programme's contribution to strengthening healthcare professionals' capacity to recognise early signs of childhood cancer and initiate timely referral for specialised treatment.

Methods: Eight one-day training sessions were conducted across six major hospitals in Bangladesh, led by senior paediatric oncology specialists. Core content covered early detection, tumour-specific symptom recognition, available treatment pathways, cancer-related services, and the importance of early referral. As part of the wider programme evaluation, a qualitative assessment was conducted to evaluate the training's effectiveness. Semi-structured interviews were conducted with a purposive sample representing approximately 10% of all training participants, and thematic analysis was used to explore learning outcomes and the application of skills in real clinical settings.

Results: The training effectively addressed key gaps in early childhood cancer detection across clinical roles and successfully trained 483 doctors and medical students across six medical facilities. Participants reported improved ability to recognise signs such as unexplained abdominal discomfort or bleeding as possible malignancies and agreed that early identification has the potential to improve childhood cancer survival by 70–90%. Several respondents described applying EWSS principles in their clinical settings, noting increased confidence in initiating early referrals.

Conclusion: EWSS training significantly strengthened healthcare professionals' ability to recognise early cancer symptoms and improved their readiness to act on potential warning signs. The training demonstrated clear qualitative impact while highlighting opportunities to strengthen training duration and reinforcement materials to improve long-term effectiveness.

Abstract 436

Development of Novel Natural Killer Cell Therapy Using a Unique NK Cell Subset

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Purpose: With novel three-stage expansion protocol that enables feeder-free, and sorter-free expansion of a unique NK cell subset (apexNK®) derived from the blood, we engineered several targeted NK cell therapies, including ACC® antibody-conjugated and chimeric antigen receptor (CAR)–modified apexNK® cells.

Methods: Antibody-conjugated apexNK cells were generated using an improved ACC® chemical conjugation (Acepodia). To address unmet clinical needs in solid tumors and to target cancer stem cells, we also developed three CAR-NK therapies— OGD2, ESCO2, and Globo H CAR-NK—using high viral transduction technology.

Results: Antitumor activity of ACC®-NK was shown with i) cells derived from healthy donor blood; ii) antibody conjugation is achieved via non-viral method; and iii) manufacturing process is significantly shortened. Besides, CAR-NK products targeting glycan antigens and cancer stem cells have been generated.

Conclusion: The apexNK® platform enables efficient expansion of a unique NK subset, achieving higher expansion than other methods. It supports development of antibody-conjugated NK therapies and CAR-NK cell products, providing a promising strategy for the treatment of hematologic malignancies and solid tumors.

Abstract 437

Ginkgetin Alleviates Cisplatin-Induced Muscle Atrophy via Inhibition of the Macrophage cGAS-STING Pathway

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Purpose: Chemotherapy-induced muscle atrophy is a severe side effect, impairing patients' quality of life and overall survival. However, the persistence of muscle atrophy in cancer survivors long after treatment completion suggests that it is driven not only by the agent's direct toxicity, but also by a persistent, chemotherapy-induced pathological immune microenvironment. Elucidating the interplay between chemotherapy drugs, the immune microenvironment, and muscle cells is essential for identifying mechanisms and potential therapeutic targets.

Methods: In this study, we investigated the critical role of macrophages in potentiating cisplatin-induced muscle atrophy by identifying a novel "amplification effect". The mechanism of the cisplatin-macrophage-muscle cell axis was validated in an in vivo mouse model of cisplatin-induced muscle atrophy.

Results: Specifically, conditioned medium from cisplatin-activated macrophages synergized with cisplatin to induce severe myotube atrophy. We identify that cisplatin activates the cGAS-STING pathway in macrophages by inducing cytosolic DNA leakage, which drives their M1 polarization and pro-inflammatory cytokines release. The pro-inflammatory microenvironment amplifies the myotoxicity of cisplatin and promotes severe muscle atrophy. Notably, ginkgetin reverses the cisplatin-induced inflammatory microenvironment by binding to the STING protein within macrophage.

Conclusion: These findings suggest that the macrophage cGAS-STING pathway is a key common mechanism and a broad-spectrum therapeutic target for treating chemotherapy-induced muscle atrophy. Collectively, this study elucidates the critical role of macrophage-mediated microenvironment in cisplatin-induced muscle atrophy, thereby providing a promising therapeutic target for chemotherapy-induced muscle atrophy.

Abstract 438

Testicular transposition as a fertility preservation measure before radiotherapy of the perineum : with report of six cases

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Purpose: With advances in comprehensive cancer treatment modalities such as surgery and radiotherapy, the clinical cure rate of childhood/adolescent cancers has increased significantly, which has led to a growing concern about impaired fertility after cancer treatment. In male children, the incidence of oligospermia increases significantly to more than 90% when the average testicular radiation dose is high at 1 Gy, and testicular translocation is a possible form of protection in such cases.

Methods: We report three cases of children who underwent testicular translocation, in which the patients' testes were moved out of the radiotherapy exposure field.

Results: At the end of radiotherapy, the testes were moved back. After radiotherapy, the testicle was returned. There was no reduction in testicular volume and the testosterone levels are still normal.

Conclusion: The results suggest that testicular transposition is safe and feasible, and its long-term effect is worthy of expectation.

Abstract 439

Epidemiological Assessment of Parvovirus B19 Genome Frequency Among Healthy Individuals and Blood Donors in Uzbekistan

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Purpose: Parvovirus B19 infects hematopoietic tissue and poses a serious risk to immunocompromised and hematological patients. Blood-borne transmission is a concern. This study evaluated the frequency of PV B19 genome detection in apparently healthy individuals and blood donors.

Methods: The study included 322 apparently healthy individuals, including 105 blood donors, examined in Uzbekistan (2013–2016). PV B19 was detected by ELISA and PCR using commercial kits. Statistical analysis was performed with OpenEpi 2009 (v2.3).

Results: PCR revealed higher PV B19 detection in apparently healthy adults versus children (<3.5 yrs: 2.4-fold; 3.5–12 yrs: 1.5-fold). Rates increased in older groups, peaking at 55–75 yrs. Blood donors showed one-third higher prevalence, underscoring the need for PV B19 screening.

Conclusion: 1. The frequency of PV B19 genome detection among apparently healthy individuals increases with age and correlates with elevated anti–PV B19 IgM levels. 2. The highest detection rates of genetic markers of parvovirus infection were observed in the age group most suitable for blood donation.

Abstract 440

When Hope Meets Reality: Parental Experiences of Pediatric Brain Tumor Care in a Resource-Limited Radiation Oncology Setting

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Purpose: In LMICs, pediatric brain tumor radiotherapy occurs amid limited imaging, technology, and prognostic uncertainty. Parents often expect cure, while clinicians must discuss risks of neurocognitive injury and disability. Parental experiences in this setting remain underexplored.

Methods: We conducted a qualitative study at PIMS Islamabad using interpretive phenomenology and constructivist grounded theory. Purposeful sampling included parents and neuro-oncology clinicians. Data from interviews, observations, and care mapping were analyzed thematically.

Results: Diagnosis was experienced as life-altering, with radiotherapy symbolizing hope and fear. Parents described distress from toxicities, neurodevelopmental decline, prolonged treatment, and financial strain. Clinicians reported imaging limitations and challenges in prognostic communication.

Conclusion: Parental experiences reflect a fragile balance between hope, uncertainty, and acceptance. Improving family-centered communication, psychosocial integration, and diagnostic–therapeutic coordination is essential for compassionate pediatric radiotherapy in resource-limited settings.

Abstract 441

Prophylactic Levofloxacin in Pediatric Oncology Patients Undergoing Chemotherapy: A Randomized Controlled Study from a Tertiary Care Center in Pakistan

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Purpose: Infections remain the leading cause of treatment failure and mortality in pediatric oncology in LMICs. Antibacterial prophylaxis is underused due to AMR concerns and system constraints. Using the Donabedian Model, we assessed whether process optimization improves outcomes.

Methods: In this prospective open-label RCT, children aged 1–14 years (N=90) with malignancies were randomized 1:1 to receive oral levofloxacin during

neutropenia (ANC <500/µL) or standard care. FN incidence was the primary endpoint.

Results: FN occurred in 32% of the levofloxacin group versus 54% of controls (RR 0.50; p<0.001). Gram-negative bacteremia and ICU admissions were reduced, and hospital stay shortened by four days. No grade 3/4 toxicity or QTc prolongation occurred.

Conclusion: Levofloxacin prophylaxis is a safe, feasible, and equity-oriented intervention that significantly reduces infectious morbidity and resource utilization in LMIC pediatric oncology, offering a scalable strategy to narrow global survival disparities.

Abstract 442

Digital Passport of the Pediatric Oncology and Hematology Service as a Decision-Support Tool for Healthcare Management

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Purpose: Digital health systems generate growing clinical and organizational data. We report tools for trend and visual analytics, quality assessment in regional pediatric oncology and hematology services, and referral planning when mandatory quality criteria cannot be met locally.

Methods: Routine digital tools were deployed: a Digital Regional Questionnaire and a Quality Assessment System, integrated into the Digital Passport for Pediatric Oncology and Hematology. State-registered software updates regional data for monitoring and decision support.

Results: The system ranks providers and regions, tracks quality trends over time, and supports referral planning in pediatric oncology and hematology when local criteria are unmet. Interoperability with the Dmitry Rogachev Center registry enables continuous data updates.

Conclusion: The Digital Passport improves monitoring of pediatric oncology/hematology, enables region benchmarking, and supports process optimization and referral routing. Integration with external registries is feasible, ensuring continuously updated data for management.

Abstract 443

ASSESSMENT OF DISTRESS AND ADAPTATION DISORDERS IN HSCT: PILOT DATA

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Purpose: Hematopoietic stem cell transplantation (HSCT) is often the only curative treatment for severe oncological, hematological, and immunological diseases. The procedure is associated with high toxicity, prolonged isolation, and complex recovery, while many patients enter HSCT after multiple relapses and significant psychological burden. Early identification of anxiety and adaptation disorders is essential for maintaining treatment adherence and improving outcomes.

Methods: A pilot study of a program for the prevention of anxiety and adjustment disorders (F41, F43) in HSCT patients was conducted in 2024–2025 at a national pediatric transplant center in collaboration with a mental health institute. Clinical-psychopathological, clinical-biographical, experimental-psychological, and statistical methods were used.

Results: The study included 43 patients (26 males, 17 females) aged 3–27 years (median 12.7) with oncological, oncohematological, hematological, and immunological diseases, as well as legal representatives of minors. Patients were stratified into three age groups: 3–7 years (n=9), 8–17 years (n=26), and 18–35 years (n=8). Distress severity before and after HSCT was assessed using the NCCN Distress Thermometer; symptoms with the Children's HSCT Symptom Questionnaire; depression with the Children's Depression Questionnaire; pain with a visual analog scale; and quality of life with PedsQL 4.0. Severe distress was identified as a significant risk factor for reduced treatment adherence and for anxiety and adjustment disorders during HSCT. Most parents lacked effective coping strategies; intolerance of uncertainty—particularly fear of death or loss of the child—was strongly associated with anxiety and helplessness. Prognostic uncertainty was the dominant parental concern.

Conclusion: High levels of distress and adaptation problems were observed in both patients and families during HSCT, negatively affecting adherence and quality of life. Early psychoprophylactic interventions and structured psychological support for families are essential components of comprehensive transplant care.

Abstract 444

The experience of using indocyanine green in pediatric thyroid surgery

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Purpose: Hypoparathyroidism one of the most common complication in pediatric thyroid surgery. This condition leads to decrease quality of patient's life. The existing method of assessment of parathyroid glands functional condition is interoperative visual examination. Unfortunately, the results of this method is not satisfied. According to several investigations the rate of the hypoparathyroidism after surgery on the thyroid gland in children is about 30%. The new method of determination of the parathyroid glands and their perfusion level by indocyanine green uses in adult patients. In our Centre we started the research of the applicability of this method in children.

Methods: Between 2025 and 2026 110 patients under the age of 18 were injected with indocyanine green during thyroid surgery. The location and perfusion of the parathyroid glands were assessed 30 seconds after injection. To determine the state of blood flow, we used a special score scale. Evaluation of parathyroid glands perfusion after indocyanine green injection is possible was estimaned by coloring organs in one of three colors (white, grey and black). The each color has a score in points (2, 1 and 0 appropriately).

Results: According to our observations the indocyanine green can be used as a navigator to determine the position of the parathyroid glands in children with benign thyroid diseases. Assessment of the blood flow of the parathyroid glands and the prognosis of their function using indocyanine green is possible for all types of thyroid tumors. According to the results of our study we can declare that in patients with at least one "white" parathyroid gland during the probe the hypoparathyroidism in the post thyroid surgery period does not occur.

Conclusion: The use of indocyanine green in pediatric thyroid surgery may contribute to early detection of intraoperative damage of the parathyroid glands. Due to this, treatment of hypoparathyroidism may be started earlier.

Abstract 445

Reconstruction of Mandible in Children: Free Flap Reconstruction or Expandable Prosthesis?

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Purpose: The most common causes of extensive defect of the mandible in the pediatric population are trauma, tumors, or congenital malformations. There are different strategies of reconstruction, including using free flaps and prostheses, in order to recover functions of chewing, speaking, and an acceptable appearance, but none of them is universally accepted. This study aimed to provide an update on aesthetic and functional outcomes in pediatric patients who undergo mandible reconstruction after having extensive bone defects.

Methods: Between 2015 and 2025, 70 children with mandible defects (mean age, 12 years; range, 2-18 years) underwent surgery to reconstruct the lower jaw with a free fibular flap (n=50) or expandable endoprosthesis (n=20) because of malignant (n=25) or benign (n=43) tumors or hemifacial microsomia (n=2). The results were evaluated from the position of facial symmetry and functional rehabilitation, including the possibility of dental implantation.

Results: The best results of free flap reconstruction were achieved in the older patient group (11-18 y.o.), while in younger children (2-10 y.o.) free flaps did not show sufficient growth to compensate the enlargement of the unaffected side, which resulted in increased facial asymmetry and the impossibility of dental implantation because of the small volume of the bone part of the flap. Expandable prostheses showed better results in younger children, resulting in better facial symmetry during their growing up and preserving the donor area for possible free flap reconstruction when they reach the age of 11-14 years.

Conclusion: Microsurgical free flap reconstruction is preferable for children aged 11-18 years, giving good aesthetic results and enabling full functional reconstruction; in younger children, better results are achieved when expandable prostheses are used, as they can be corrected in size during the children's active growth phase.

Index by Cancer Type & Topic

Cancer types, treatment modalities and clinical topics covered in this Abstract Book, with page numbers.

A	
Acute Lymphoblastic Leukaemia (ALL)	**4, 5, 6, 8, 10, 11, 13, 15, 16, 17, 18, 20, 21, 24, 25, 28, 29, 30, 31, 32, 33, 84**
Acute Myeloid Leukaemia (AML)	**7, 8, 15, 18, 19, 21, 22, 23, 24, 30, 31, 32**
Aplastic Anaemia	**4**
Atypical Teratoid / Rhabdoid Tumour (AT/RT)	**42**
B	
Biomarker	**5, 11, 19, 20, 46, 68, 69, 87**
Bone Marrow	**5, 6, 7, 8, 10, 11, 12, 13, 15, 16, 17, 19, 20, 21, 23, 27, 28, 29, 31, 32, 40, 47, 48, 50, 54, 58, 69, 84, 85**
Brain & CNS Tumours	**35, 36, 38, 43**
C	
Chemotherapy	**6, 7, 8, 9, 10, 12, 13, 14, 15, 16, 17, 18, 21, 23, 25, 26, 27, 29, 30, 32, 33, 34, 35, 36, 37, 39, 40, 41, 42, 43, 44, 45, 46, 48, 49, 51, 52, 53, 54, 55, 56, 57, 58, 59, 60, 61, 62, 63, 64, 65, 66, 67, 68, 70, 71, 72, 73, 74, 75, 78, 79, 82, 83, 87, 90, 91, 93, 96, 97**
Chronic Myeloid Leukaemia (CML)	**23**
E	
Embryonal Tumours (ETMR)	**35, 36, 37, 55**
Ependymoma	**41, 42**
L	
Langerhans Cell Histiocytosis (LCH)	**6, 7, 8, 22, 23, 26**
Leukaemia	**4, 5, 6, 7, 8, 9, 10, 11, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 28, 29, 30, 31, 33, 34, 84**
Low- & Middle-Income Countries	**17, 26, 29, 30, 36, 38, 43, 54, 62, 73, 77, 81, 83, 85, 87, 89, 97**
Lymphoblastic Lymphoma	**8, 29**
Lymphoma	**8, 10, 12, 14, 15, 18, 19, 20, 21, 23, 25, 26, 28, 29, 32, 82, 87**
M	
Medulloblastoma	**37, 38, 39, 40, 41, 43, 83**
N	
Nasopharyngeal Carcinoma	**44, 47, 75**
Neuroblastoma	**46, 47, 48, 50, 51, 52, 54, 55, 56, 58, 59, 61, 63, 66, 67, 69, 72, 74, 88, 94**
Non-Hodgkin Lymphoma	**15, 18, 20**
Nursing	**77, 78, 79, 85, 88, 90, 91, 92, 93**
Nutrition	**32, 48, 81**
O	

Epidemiology	35, 37, 52, 56, 67, 81, 86, 88, 89, 90, 94, 97
Ewing Sarcoma	40, 48, 59, 63, 64, 65
G	
Genetics / Genomics	4, 6, 7, 9, 11, 12, 17, 21, 22, 24, 26, 28, 29, 31, 36, 37, 39, 40, 42, 43, 48, 51, 52, 53, 54, 55, 64, 66, 69, 76, 78, 83, 89, 95, 97
Germ Cell Tumour	36, 60, 64, 67, 70, 71, 75
Glioma / Glioblastoma	37, 40, 42, 44
Glioma — High-Grade	36, 37
Glioma — Low-Grade	44
Graft-versus-Host Disease (GvHD)	4, 5, 27, 29
H	
Haematopoietic Stem Cell Transplantation	5, 6, 19, 22, 23, 27, 29, 30, 31, 33, 34, 35, 48, 50, 55, 70, 77, 94, 95
Hepatoblastoma	49, 52, 58, 66, 68, 70, 74, 75
Hepatocellular Carcinoma	67
Histiocytosis / LCH	6, 7, 8, 17, 22, 23, 26, 33
Hodgkin Lymphoma	12, 15, 18, 19, 20, 21, 23, 25, 26, 82, 87
I	
Osteosarcoma	61, 63, 68, 71
P	
Pain Management	78, 85, 93
Palliative Care	30, 32, 77, 85, 91, 93, 95
Q	
Quality of Life	9, 31, 33, 43, 50, 52, 56, 68, 71, 74, 81, 85, 88, 89, 92, 93, 94, 96, 97
R	
Radiotherapy	12, 21, 36, 37, 39, 40, 42, 43, 44, 48, 51, 52, 53, 54, 55, 56, 57, 58, 61, 62, 63, 64, 66, 67, 69, 74, 75, 78, 84, 96, 97
Retinoblastoma	34, 35
Rhabdomyosarcoma	45, 49, 52, 53, 55, 63, 66, 68, 69, 71
S	
Sarcoma	40, 48, 59, 61, 63, 64, 65, 66
Soft Tissue Sarcoma	44, 55, 61, 65, 66, 68
Surgery	11, 22, 27, 35, 36, 37, 38, 39, 40, 41, 42, 43, 44, 45, 46, 47, 48, 49, 51, 52, 53, 54, 55, 56, 57, 58, 59, 60, 61, 62, 63, 64, 65, 66, 67, 68, 69, 70, 71, 72, 73, 74, 75, 78, 79, 81, 86, 95, 96, 98
Survivorship	24, 25, 33, 36, 61, 74, 80, 81, 83, 84, 94, 96
T	

Immunotherapy	5, 12, 19, 22, 24, 33, 36, 46, 48, 50, 51, 54, 58, 59, 63, 64, 75
Infection / Sepsis	4, 5, 6, 7, 14, 15, 16, 17, 18, 22, 27, 29, 30, 38, 40, 52, 58, 61, 68, 77, 79, 80, 85, 86, 89, 91, 92, 94, 97

K

Kidney / Renal Tumours	13, 14, 30, 44, 46, 49, 55, 57, 62, 64, 72, 74, 75, 76, 79, 83, 88, 89, 90, 94

Targeted Therapy	8, 9, 20, 21, 24, 26, 37, 43, 44, 47, 51, 59, 60
Thyroid Cancer	44, 47, 75

W

Wilms Tumour / Nephroblastoma	48, 49, 54, 55, 57, 71, 72

Index by Author

Abstracts listed by first author (alphabetically by surname). Authors with more than one abstract show all abstract numbers separated by commas.

A

Nigora Abdurahmonova et al. 187
Nazgul Abulkhanova et al. 408
Mai Adel et al. 414
Bishow Nath Adhikari et al. 385
Alia Ahmad et al. 224, 415
Sh. Ainur[1] et al. 282
Eray Akay 386
Hiba Al-Tarrah 366
Gunel Alaskarova 139
Maria Aleksandrova et al. 425
Teimur Aliev et al. 262, 263, 345
Praeploy Amornsillaphachai et al. 376
Wenbin An et al. 168
Bambang Ardianto et al. 142
Eggi Arguni et al. 140
Sonali Arora et al. 197, 380
Jasni Ashraf et al. 226
Yin May Aung et al. 157
Elen Avetisyan et al. 123
Aamir Aslam Awan et al. 109, 149, 158
Burca Aydin et al. 169
Ezozakhon Azimova et al. 342
Kevin El Azzi et al. 372

B

Suhani Barbhuiyan et al. 206
Hasan Batis 246
Mariell Vera C. Bañaga et al. 144
Prateek Bhatia et al. 102
Olga I. Bogdanova et al. 270
Konstantin Boiko et al. 238, 239
Irina Borovikova 384
Natalia Burlaka et al. 198

C

Jiabin Cai et al. 360
Angelo Clarissa Cantalejo 148
Narendrakumar Chaudhary et al. 120
Temuulen Munkhdalai et al. 416
Hsu Myint Myat et al. 309
Naga Anudeep Mylavarapu et al. 196, 379

N

Khongorzul Nasanjargal et al. 352
Linh Thi My Nguyen et al. 118
Tung Nguyen 290
Mai Cam Giang Nguyen[1] et al. 124
Tran Thi Lien Nhi et al. 147
Xin Ni 252
Lesley Ann Nifras-Tiu 138
Huizhong Niu et al. 305, 353
Maria Noelyn NOCOM-CUEVASNocom-Cuevas et al. 291
Erdenetsetseg Nyamsuren et al. 417

O

Enkhee Ochirjav et al. 240
Isaac Ogola et al. 405
Rj Laine C. Olandia-Luna et al. 393
Nur Olgun et al. 260
Anna Teresa Olmedo et al. 391
Amiel Orcajada et al. 319
Gokcen Tugce Ozsut et al. 250

P

Ekata Pal et al. 283
Ruzanna Papyan et al. 371
Veerendra Patil et al. 202
Tatyana Pavlova 133
Xiaomin Peng et al. 268
Nitin James Peters et al. 347
Dang Anh Thu Phan 167
Trang Phan et al. 113
Kshounish Pradhan et al. 129
Irina Protsenko et al. 362

Q

Mohammed Sajid Qureshi et al. 332, 348
Samreen bano Sajid Qureshi et al. 301

R

Serafima Chechelnitskaya et al.	392
Haipin Chen et al.	161
Xia Chen et al.	101
D	
An Dang et al.	324
Sushenkova Daria et al.	232
Adriana Davtyan et al.	125
Jun Deng et al.	423
Li'an Du et al.	185
Chao Duan	252
Enkeleda Duka et al.	266
Daniil Dzhavadov et al.	177, 179, 180, 181, 182
E	
Anna Elfimova et al.	343
Indira Erimbetova et al.	132
Ekaterina Evstigneeva et al.	400
F	
Hira Faheem	418
Jianming Fang	295
Roula Farah et al.	245, 368, 426
Ana Farooq	403
Shaoguang Feng et al.	334
Fernandez et al.	229
Andrey Flegontov et al.	228
Libing Fu	252
Qiang fu et al.	233
G	
Alexander Galimov et al.	152
Gankhuu Gankhuyag et al.	114
Lu Gao et al.	153
Ya Gao et al.	251
G.M. Goibova et al.	235
Elena Golovina et al.	166
Han Gong	295
Elena Gorokhova et al.	310
Hiroaki Goto et al.	365
Nikolay Grachev et al.	258, 314, 444, 445
Miner Gu et al.	112
Rendi Prawira Gunawan et al.	156
H	
Gavriil Rabaev et al.	359
Nussupova Raigul et al.	236
Mark Dan S. Ranches	293
Justine Andrea Recinto	317
Shravani Reddy et al.	225
Feruza Rizaeva et al.	439
Sibel Rizaeva	406
Anastasia Rodina et al.	339
Ekaterina Romanenko et al.	194
Olga Romantsova	320, 321, 322, 323
Pritam Singha Roy et al.	200, 201
Desy Rusmawatiningtyas et al.	399
Khilola Rustamova et al.	183
Diana Rybakova	363
S	
Dilnasheen Safdar	424
Nur Melani Sari et al.	389
Melati Arum Satiti et al.	137
Hermine Sayiyan et al.	127
Emre Sefa et al.	269
Hannah Grace Segocio et al.	294
Evgeniy Senchurov et al.	337
Karina Sergeenko et al.	344
Hijab Shaheen	440, 441
Saurav Sharma et al.	300, 374
Ulyana Sheleh et al.	410
Liang Shi	155, 280
Zhang Shouhua et al.	284
Zhu Shuai[1] et al.	281
Anvar Shukullaev et al.	325
Gianhope T. Silao	297
Deependra Singh et al.	378
Minu Singh et al.	370
Elena Skorobogatova et al.	188
Mariia Skvortsova et al.	261
Aleksei Slinin et al.	442
Darya Smirnova et al.	195
Lily Smirnova et al.	311
Munguntsetseg Sodnom	276
Has Sothearak et al.	192

Krisna Handayani et al.	122
Micaella Hansen et al.	422
Munlima Hazarika et al.	131, 401
Guoqian He et al.	285, 289
Min He et al.	346
XinYu He	295
Fandi Hendrawan et al.	222
Trang Hoang et al.	265
Julieta Hoveyan et al.	219
Saten Hovhannisyan et al.	163
Junting Huang et al.	356
Niu Huizhong	277
I	
Sapura Ibragimova et al.	143
Sobirjonov Islom et al.	189, 190
Nikolay Ivanov et al.	256
A.A. Ivanova et al.	383
J	
Richa Jain et al.	107
Dhaarani Jayaraman	212
Dapeng Jiang	350, 438
Dapeng Jiang et al.	437
Wenxuan Jiang et al.	111
Fenfen Jin et al.	207
Liu Jing et al.	279
Zhang Jingying	173, 174, 175
Dipika Joshi	431, 435
K	
Dana Kanaan et al.	404
Md Anwarul Karim et al.	136
Igor Kasich et al.	220, 237
Tomomi Kawakami et al.	394
Ilya Kazantsev et al.	271
Li Kehao et al.	299
Kh.A.Aleskerova et al.	288
Rina Khabarova et al.	407
Zukhra Khashimova et al.	267
Udval Khurelbaatar et al.	302
Hoa Thi Kim et al.	199
Denis Korneev et al.	244
Irina Kostareva	211
Dennis Tak-loi Ku et al.	221
Nara Stepanyan	210
Yan Su	252
Yuan Sun	295
Eddy Supriyadi et al.	135
Tarun Kumar Suvvari	412
Tarun Kumar Suvvari et al.	413
T	
Robert Tadevosyan et al.	128, 432
Marlo Tampon et al.	398
Leonard Tan et al.	164
Ru Tang et al.	150
Suoqin Tang	295
Murod Tashmetov et al.	338
Margarita Teleshova et al.	286
Galina Tereshenko et al.	116
Kok Hoi The	409
Yin Myat Thu et al.	307, 308
Zhi Tian	326, 361
Dragana Stanic Tisma et al.	390
Marya Titova et al.	230, 231
Polina Tolkunova et al.	217, 223
Alexander Tretyakov	396
Maharshi Trivedi et al.	115, 264
Irina Trushkova et al.	193
Tsengelmaa.Ch et al.	213
Bayarbat Tsevegjav et al.	172
Sainmaa Tsogtbayar et al.	364
El Nino Tunjungsari et al.	420
Mukhammadi Turdiev et al.	119
U	
Natalya Uskova et al.	349
Hermin Usman et al.	377
Dinara Utalieva et al.	358, 430
V	
Timur Valiev et al.	160
Dheeraj Vanama et al.	388
Anastasiia Volkova et al.	205
W	
Kathleen Stephanie Wahing et al.	170
Yang Wan et al.	367
Chen Wang et al.	335
Huanmin Wang	387

Svetlana A. Kulyova et al.	247
Svetlana Kulyova et al.	336
Narendra Kumar et al.	117
Oleg Kuznetsov	178
L	
Catherine G. Lam et al.	381, 382
Nguyen Lan et al.	249
Zach Lee et al.	428
Siyu Lei et al.	340, 341
Wu Lei et al.	278
Leran Li	151
Felix Liauw et al.	145, 303
Xinrong Lin et al.	253
Hong Yan Liu et al.	296
Hongyan Liu	215, 234, 295, 298
Jiale Liu et al.	304
Lipeng Liu et al.	103
Nan Liu	104
Qi Liu	295
Rongrong Liu et al.	159
Siying Liu et al.	154
Weiguang Liu et al.	333
Xiaoning Liu et al.	227
Yukai Liu	295
Zhikai Liu	252
Baskhuu Lkhagvasuren	411
C-Khai Loh et al.	397
M	
Yumiao Mai et al.	313
Emil Makimbetov et al.	402
Maksim Maksimov	243
Cindy Faye Marquez et al.	171
Leni Mathew et al.	146
Dilfuza Matkarimova et al.	184
Vu D.U.V. Md et al.	121
Nikolay Merkulov	316
Aleksey Metelin et al.	357
Jing Miao et al.	208
Valeria Migunova et al.	242
Takashi Mikami et al.	204
Juan Wang et al.	255
Wenxia Wang et al.	110
Xingchen wang et al.	130
Yuchi Wang	295
Zhixuan Wang et al.	273, 275
Linnan Wu et al.	355
Shengchao Wu	162
X	
Weiji Xie et al.	257, 312
Xilin Xiong et al.	287
Xiaojun Xu	209
Changwen Xue et al.	429
Y	
Su Yadana et al.	373
Weiling Yan et al.	203
Peiyi Yang et al.	218
Wei Yang	351
Minzhi Yin	318
Tang Yinbing	272
Alice Yu et al.	315
John Yu et al.	436
Tong Yu	252
Xiuli Yuan	259
Idha Yulandari et al.	141
Z	
Huma Zafar et al.	214, 216, 369, 427, 433
Bai Johanna Zainal et al.	395
Nur Farahin Zakaria	421
Dr.Ainaeek Zaman et al.	134
Haixia Zhang	165
Jianguo Zhang et al.	292
Lei Zhang et al.	254
Shihan Zhang	248
Weiling Zhang	295
Yi Zhang	327
Yi Zhang et al.	328, 329, 330
YingJin Zhang et al.	108
Yingli Zhang	295
Zihan Zhang et al.	106
Wen Zhao	252

Volha Mishkova et al.	**105, 434, 443**
Vera Mkhitaryan	**419**
Potturu Monikha et al.	**354**
Jessa Montejo-Go	**176**
Sardor Mukhammadiev et al.	**186**
Sherzodbek Mukhammadjonov	**191**
Wen Zhao et al.	**331**
Olga Zheludkova et al.	**241**
Qipeng Zheng et al.	**306, 375**
Zhencang Zhou	**126**
Yongbing Zhu et al.	**274**

SOCIÉTÉ INTERNATIONALE D'ONCOLOGIE PÉDIATRIQUE

SIOP

INTERNATIONAL SOCIETY OF PAEDIATRIC ONCOLOGY

Established in 1969, the International Society for Paediatric Oncology is the only global multidisciplinary society entirely devoted to paediatric and adolescent cancers. With a steadily growing membership, SIOP currently unites over 4,000 healthcare professionals and researchers working with paediatric patients worldwide. The community shares a strong common purpose: to increase survival rates, to improve quality of survival and to ensure care for all.