



第六屆St. Jude-VIVA- 國家兒童醫學中心(上海) 血液/腫瘤國際會議

6th St. Jude-VIVA-NCMC (Shanghai)
Paediatric Haematology/Oncology Forum

Hong Kong Children's Hospital 香港兒童醫院

8-9 November 2025



Welcome Messages

Welcome message by Professor Ching-Hon PUI, St. Jude Children's Research Hospital

It is with immense pleasure that we welcome you to the 6th Annual St. Jude-VIVA-National Children's Medical Centre (Shanghai) Forum, a premier platform for collaboration, innovation, and education in paediatric oncology across Asia. The Forum continues to expand its global reach, fostering greater engagement and knowledge sharing across borders.

This Annual Forum stands as a testament to the enduring partnership between the VIVA China Children's Cancer Foundation Limited, St. Jude Children's Research Hospital, and Shanghai Children's Medical Centre. Under the inspired leadership of Dr. Carlos Rodriguez-Galindo, this collaboration has propelled advancements in childhood cancer education, research, and patient care throughout Asia and beyond.

The scientific committee has curated a comprehensive two-day program covering four major areas in paediatric oncology: leukaemia; solid and brain tumours; blood and bone marrow transplantation; and cancer survivorship. We are honoured to feature lectures from leading international experts. Day 1 will focus on recent advances in ALL, new insights into biology, diagnosis, and management of T-cell ALL, T-cell immunity and treatment outcomes, cellular therapy innovations, strategies to address global disparities in cancer care, advances in high-risk neuroblastoma, personalized approaches for low-grade gliomas, survivorship care, and biologic mechanisms underlying neurocognitive impairment in ALL survivors. Day 2 will address management of low- to intermediate-risk neuroblastoma, autologous haematopoietic stem cell transplantation in solid tumours, treatment of brain tumours, challenges faced by brain tumour survivors, and will conclude with the VIVA-Asia Blood and Marrow Transplant meeting.

I would like to express my heartfelt gratitude to the VIVA China Children's Cancer Foundation Limited, led by its visionary founder, Mrs. Jennifer Yeo, and Beelan Tan for their unwavering commitment and tireless fundraising efforts in support of our study group. My sincere thanks also go to our generous sponsors, distinguished speakers, and the dedicated organizing committee, including Professor CK Li, Professor Godfrey Chan, Dr. Alex Leung, Dr. Frankie Cheng, Dr. Daniel Cheuk, Dr. Dennis Ku, Dr. Yin Ting Cheung, and many others—whose enthusiasm and hard work have made this Forum possible.

Thank you all for joining us in this vital mission. We hope the 6th St. Jude-VIVA-National Children's Medical Centre (Shanghai) Forum will inspire, educate, and empower you, and we look forward to your active participation in the exciting programs ahead.



Professor Ching-Hon PUI, M.D.
Professor, St. Jude Children's Research Hospital



Welcome Messages

Welcome message by Mrs. Jennifer YEO, Chairperson of VIVA China Foundation

Welcome to the 6th St. Jude-VIVA-NCMC Paediatric Haematology/Oncology Forum. This year marks the first time it is organized and held in Hong Kong.

The Forum is associated with the Chinese Children Cancer Group-Acute Lymphoblastic Leukaemia (CCCG-ALL) Study Group that started in China. VIVA CHINA has supported the CCCG-ALL Study Group since the first meeting on a Sunday during Golden Week in 2014 in Shanghai at Shanghai Children's Medical Centre, attended by leaders from Jiaotong University and Shanghai Children's Medical Centre (SCMC), Dr Ching-Hon Pui of St Jude Children's Research Hospital, Minister Chen Zhu, the former Health Minister and Vice Chair of the Standing Committee of the National People's Congress of China, doctors representing the first 20 hospitals of the Study Group and my friends Tan Bee Lan, KK Chua and me. Soon after, my friends and I went on to incorporate VIVA China as a Hong Kong charity to fund this Study Group. I remember with a thankful heart that although Hong Kong at that time had outstanding results for the treatment of ALL, it still joined the Study Group and, led by Dr Li Chi-Kong of the Chinese University of Hong Kong who represented all the hospitals in Hong Kong engaged in the treatment of paediatric ALL, it remains an active and committed member until today.

The CCCG-ALL Study Group is the brainchild of Dr Ching-Hon Pui. Without him, it would not have been born. Dr Pui has visited China regularly for the past 35 years to support paediatric oncologists in treating children with ALL. More than eleven years ago, when he shared with me his dream to raise the cure rate in China, I did not hesitate to support him and until now, VIVA China considers it its honour to fund the budget for the Study Group. Today the Study Group is one of the biggest in the world both in number of patients enrolled and in homogeneity of the patients, with 16,000 children from 26 hospitals in China. Central data collection, verification, analysis and reporting continue to be managed by Dr Cai Jiao Yang at SCMC which has played a major role in contributing to the success of the Study Group as evidenced by the high survival rate achieved and the publication of more than twenty papers in international medical and scientific journals. This places China doctors and scientists on the world stage and enables them to share their knowledge with the rest of the world.

I thank the Hong Kong organising committee led by co-chairs Dr Li Chi Kong from the Chinese University of Hong Kong, Dr Frankie Cheng from Hong Kong Children's Hospital Haematology and Oncology Centre and Dr Alex Leung from the Hong Kong Paediatric Haematology and Oncology Study Group, for their extraordinary efforts to raise funds and organise this Forum; Dr Pui for his transformative leadership, hard work and continuing commitment; Zhang Hao, President of SCMC and Jiang Zhong Yi former President of SCMC for their continuous support; Dr Godfrey Chan (the University of Hong Kong) of the VIVA China Advisory Board; VIVA China's partner, VIVA Foundation for Children with Cancer, incorporated in Singapore almost 20 years ago, for their support and collaboration in many programmes and projects over the years. I thank the doctors and hospitals of the Study Group for their continuing dedication and devotion. I remember with gratitude Dr Tang Jing Yan of SCMC for her leadership in the early days of the Study Group. I thank the speakers and participants of this Forum for their support. Through our cooperation and respective contributions, we can surely achieve the dream to raise the cure rate of children with cancer in China, Hong Kong SAR, and ultimately in Asia and the world.



Mrs. Jennifer YEO
Chairperson of VIVA China Foundation



Welcome Messages

Welcome message by Local Organizing Committee

On behalf of the Local Organising Committee, it is our great pleasure to welcome you to the 6th St. Jude VIVA SMC (Shanghai) Paediatric Haematology / Oncology Forum, co-organised by the Chinese University of Hong Kong (CUHK), Hong Kong Children's Hospital (HKCH) and Hong Kong Paediatric Haematology and Oncology Study Group (HKPHOSG).

This forum is more than a scientific meeting—it is a celebration of collaboration. Together, we form a global community united by a shared mission: to improve the lives of children affected by cancers and blood disorders. In this forum, we will exchange knowledge, challenge ideas, and build bridges that extend far beyond this forum.

We would like to express our heartfelt gratitude to the world-renowned experts for bringing their expertise and inspiration; to our attendees for being here to learn, exchange ideas and make friends; and to our partners and sponsors for their unwavering support. Your contributions are the foundation of this forum's success and the driving force behind our continued progress.

Let this forum be a catalyst for deeper partnerships, new initiatives, and lasting friendships. May the conversations we begin here spark collaborations that endure—and may our shared efforts continue to shape the future of paediatric oncology. Thank you again for being here. We are sure the forum will be impactful, inspiring, and unforgettable to you all.



A handwritten signature of Prof Chi Kong LI.

Prof Chi Kong LI



A handwritten signature of Dr Frankie CHENG.

Dr Frankie CHENG



A handwritten signature of Dr Alex LEUNG.

Dr Alex LEUNG

Co-chairs, Local Organizing Committee
6th St. Jude VIVA SMC (Shanghai) Paediatric Haematology / Oncology Forum



香港兒童醫院
Hong Kong Children's Hospital



Local Organizing Committee

Co-Chairpersons of Local Organizing Committee

- Prof LI Chi Kong
Research Professor, The Chinese University of Hong Kong (CUHK)
- Dr CHENG Wai Tsoi Frankie
Service Head, Haematology and Oncology Centre, Hong Kong Children's Hospital (HKCH)
- Dr LEUNG Wing Kwan Alex
Chairperson, Hong Kong Paediatric Haematology and Oncology Study Group (HKPHOSG)

Local Organizing Committee (in alphabetical order)

- Dr CHAN Yau Ki Wilson
- Dr CHEUK Ka Leung Daniel
- Dr CHOW Tin Wai Terry
- Dr KU Tak Loi Dennis
- Dr LAM Kee See Grace
- Dr TONG Pui Yung Grace

General Information

Congress Venue

- **Address:** 1/F, Tower A, Auditorium and Lecture Rooms, Hong Kong Children's Hospital
1 Shing Cheong Road, Kai Tak, Kowloon, Hong Kong SAR, China
- **Tea break and lunch location:** Foyer area outside Auditorium
- **Transportation to Congress Venue**
 - One 60-seat shuttle bus departing at 8:15am at Ngau Tau Kok Road
(3-minute walk from Ngau Tau Kok MTR station Exit A)

Gala Dinner

- **Date:** 8 November 2025 (Saturday)
- **Venue:** Federal Cruise Banquet Centre, Shop N205-206, Zone B, 2/F, Kai Tak Cruise Terminal, 33 Shing Fung Road, Kowloon Bay
- **Transportation to Dinner Venue from Congress Venue (Hong Kong Children's Hospital)**
 - Four 60-seat shuttle buses
- **Transportation from Dinner Venue**
 - **To Shenzhen (provided by CUHK):** two 60-seat shuttle buses departing at 8:30pm
 - **To Kowloon Bay MTR station:** two 60-seat shuttle buses (departing at 8:30pm and 9pm)

一般信息

会议地点

- **地址:** 中国香港特别行政区 九龙启德承昌道1号 香港儿童医院A座一楼演讲厅及演讲室
- **茶歇及午餐地点:** 演讲厅外大堂
- **前往会议地点的交通**
 - 一辆60座穿梭巴士, 上午8:15从牛头角道出发 (从牛头角港铁站A出口步行3分钟)

晚宴

- **日期:** 2025年11月8日 (星期六)
- **地点:** 联邦邮轮宴会中心, 九龙湾承丰道33号启德邮轮码头二楼B区N205-206号铺
- **从会议地点(香港儿童医院)前往晚宴地点的交通:** 四辆60座穿梭巴士
- **从晚宴地点出发的交通**
 - **前往深圳(由香港中文大学提供):** 两辆60座接驳巴士, 晚上8:30出发
 - **开往九龙湾地铁站:** 两班60座接驳巴士 (晚上8:30和9:00出发)

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Programme at a Glance

8 November 2025 (Day 1)

Time	Session Title	Speaker(s)	Session Chair(s)	Location
08:30 – 09:00	Registration			
09:00 – 09:15	Opening Remarks	Dr. Ching-Hon PUI, Chair, Scientific Committee Mrs. Jennifer YEO, Director, VIVA China Children's Cancer Foundation Mrs. Ann Kung, Chairlady, Hospital Governing Committee Hong Kong Children's Hospital (HKCH)		
09:15 – 10:00	New Frontiers in the Genetics and Treatment of Childhood Acute Lymphoblastic Leukaemia	Dr. Ching-Hon PUI (St Jude Children's Research Hospital, USA)	1. Dr. Shuhong SHEN, Shanghai Children Medical Center 2. Dr. Frankie Cheng, Hong Kong Children's Hospital	Auditorium Tower A, Hong Kong Children's Hospital
10:00 – 10:40	New Insights into the Biology, Diagnosis, and Management of T-cell acute lymphoblastic leukemia and lymphoma	Dr. David T. TEACHEY (The Children's Hospital of Philadelphia, USA)		
10:40 – 11:00	Tea / Coffee Break			
11:00 – 11:40	T-cell Immunity and Its Impact on ALL Treatment Response	Dr. Jun YANG (St Jude Children's Research Hospital, USA)	1. Dr. Xiaowen Zhai, Fudan University Children's Hospital 2. Dr. Daniel Cheuk, Hong Kong Children's Hospital	Auditorium Tower A, Hong Kong Children's Hospital
11:40 – 12:20	CAR T Cell Therapy in Paediatric Oncology: Current Status and Future Prospects	Dr. Crystal L. MACKALL (Stanford Center for Cancer Cell Therapy, USA)		
12:20-13:00	Curing Children with Cancer: Strategies to Address Global Disparities in Childhood Cancer Care and Control	Dr. Carlos RODRIGUEZ-GALINDO (St Jude Children's Research Hospital, USA)		
13:00 – 14:00	Lunch			

Programme at a Glance

8 November 2025 (Day 1)

14:00 – 14:40	Advances in Haploididential Transplantation	Dr. Rupert HANDGRETINGER (Universty of Tuebingen, Germany)	1. Dr. Edwin Chan, Hong Kong Children's Hospital Dr. Dennis Ku, Hong Kong Children's Hospital	Auditorium Tower A, Hong Kong Children's Hospital
14:40 – 15:10	Recent Advances in Treatment of High-Risk Neuroblastoma	Dr. Julie PARK (St Jude Children's Research Hospital, USA)		
15:10 – 15:50	Personalized and Tailored Approach for Paediatric Low Grade Gliomas	Dr. Ibrahim QADDOUMI (St Jude Children's Research Hospital, USA)		
15:50 – 16:10	Tea / Coffee Break			
16:10 – 16:50	Supporting Adherence to Childhood Cancer Survivorship Care	Dr. Melissa HUDSON (St Jude Children's Research Hospital, USA)	1. Dr. Jiaoyang CAI, Shanghai Children Medical Center 2. Dr. Yin Ting CHEUNG, The Chinese University of Hong Kong	Auditorium Tower A, Hong Kong Children's Hospital
16:50 – 17:30	Biologic Mechanisms Associated with Neurocognitive Impairment in Survivors of Paediatric Acute Lymphoblastic Leukemia	Dr. Kevin KRULL (St Jude Children's Research Hospital, USA)		
18:00- 20:30	Gala Dinner for all participants			

Programme at a Glance

9 November 2025 (Day 2)

AM Session	Auditorium Tower A, Hong Kong Children's Hospital			Hilton Shenzhen Futian (深圳大中華 希爾頓酒店)	
08:30 – 12:30	St Jude VIVA Forum on solid tumours and survivorship				
	Topic	Speaker	Session Chair	09:00 – 12:30 CCCG ALL Study Group meeting (Closed meeting) CCCG-ALL 協作組 閉門會議	
08:30 – 09:05	Management of low / intermediate risk neuroblastoma	Dr. Julie PARK (St Jude Children's Research Hospital, USA)			
09:05 – 09:40	Autologous HSCT in childhood solid tumour	Dr. Rupert HANDGRETINGER (University of Tuebingen, Germany)	Dr. Wilson CHAN, Hong Kong Children's Hospital	Satellite online at Lecture rooms 1-2, 1/F, Tower A, Hong Kong Children's Hospital	
09:40 – 10:20	Case Discussion Neuroblastoma: Dr. Yanna MAO (Henan Children's Hospital) Dr. Evelyn LU (Hong Kong Children's Hospital)				
10:20 – 10:40	Tea / Coffee Break			Dr. Dennis KU, Hong Kong Children's Hospital	
10:40 – 11:15	Personalized and Tailored Approach for Paediatric Low Grade Gliomas	Dr. Ibrahim QADDOUMI (St Jude Children's Research Hospital, USA)			
11:15 – 11:50	Perceived challenges and support in life activity & functioning of childhood brain tumour survivors in Hong Kong	Ms. Sau Fong CHOW (Professional Services Manager, Children's Cancer Foundation, Hong Kong SAR)			
11:50 – 12:30	Case Discussion Brain tumours: Dr. Jie XU (Peking University People's Hospital) Dr. Christy MAK (Hong Kong Children's Hospital)				
	CLOSING Closing Remark: Dr. Alex LEUNG, Chairman, Hong Kong Paediatric Haematology and Oncology Study Group (HKPHOSG)				
PM Session	Lecture Rooms 1 & 2, 1 st Floor, Tower A, Hong Kong Children's Hospital		Hilton Shenzhen Futian (深圳大中華希爾頓酒店)		
	13:00 – 17:00 VIVA-Asia Blood and Marrow Transplant (VABMT) Meeting (Hybrid: Physical with Zoom)		13:00-17:00 NCMCs-LTFU (Closed meeting) NCMCs-LTFU 協作組閉門會議		

Info link for VABMT autumn school



Conference Website

**Ching-Hon PUI, M.D.**

Professor, St. Jude Children's Research Hospital

Co-Leader, Haematological Malignancies Program, American Cancer Society

Member, Fahad Nassar Al-Rashid Chair of Leukaemia Research, Oncology Department

Dr Ching-Hon PUI is a pioneering paediatric oncologist whose work has transformed treatment for childhood acute lymphoblastic leukaemia (ALL). At St. Jude Children's Research Hospital, he introduced minimal residual disease monitoring and personalized therapy, raising cure rates above 90% and eliminating the need for brain irradiation. He identified a drug-induced secondary leukaemia syndrome, prompting global changes in chemotherapy. His current research focuses on genomics and targeted therapies. Internationally, he co-founded the Ponte di Legno ALL study group and launched the St. Jude-VIVA Forum. A highly cited investigator, he served as 2019 President of the Society of Haematologic Oncology.

**David T. TEACHEY, M.D.**

Professor of Paediatrics, Garrett Brodeur, MD Endowed Chair in Oncology

Divisions of Haematology and Oncology, The Children's Hospital of Philadelphia

University of Pennsylvania, Perelman School of Medicine

Dr David TEACHEY is Professor of Paediatrics and Garrett Brodeur Endowed Chair in Oncology at the Children's Hospital of Philadelphia and University of Pennsylvania. An R01-funded physician-scientist, he leads clinical trials and translational research in paediatric acute lymphoblastic leukemia (ALL). He chairs the ALL Disease Committee for the Children's Oncology Group and co-leads CHOP's Immune Dysregulation Frontier Program. With over 200 publications and 30,000 citations, Dr. Teachey is a recognized expert in ALL and immune disorders. He has mentored numerous clinician-scientists and was elected to the American Society of Clinical Investigation and American Paediatric Society.

**Jun YANG, B.S., M.S., Ph.D.**

Director, Division of Pharmaceutical Science, Department of Pharmacy and Pharmaceutical Sciences, St. Jude Children's Research Hospital

Endowed Chair in Pharmacogenomics, Department of Pharmacy and Pharmaceutical Sciences, St. Jude Children's Research Hospital

Dr Jun YANG is Director of Pharmaceutical Sciences and Endowed Chair in Pharmacogenomics at St. Jude Children's Research Hospital. His research focuses on cancer pharmacogenomics and precision medicine for childhood acute lymphoblastic leukemia (ALL). He discovered key ALL risk loci, rare germline predisposition variants, and NUDT15 deficiency as a major cause of thiopurine toxicity—leading to FDA label changes. Dr. Yang also identified LCK dependency and developmental state as therapeutic vulnerabilities in ALL. A recipient of the NIH Outstanding Investigator Award and AACR Waun Ki Hong Award, he served as President of the Pharmacogenomics Global Research Network (2022–2023).

**Crystal L. MACKALL, B.S., M.D.**

Ernest and Amelia Gallo Family Professor of Paediatrics and Medicine,

Stanford University

Dr Crystal MACKALL is the Ernest and Amelia Gallo Family Professor at Stanford University, Founding Director of the Stanford Center for Cancer Cell Therapy, and Director of the Parker Institute for Cancer Immunotherapy. A leader in paediatric cancer immunotherapy, she has pioneered CAR T cell therapies, including CD19 and CD22 CARs, and advanced treatments for brain tumours and solid cancers. Her work on T cell biology and exhaustion has shaped next-generation immune therapies. A member of the National Academy of Medicine, she has published over 300 papers, co-founded three biotech companies, and received numerous honours for her transformative contributions to cancer research.

**Carlos RODRIGUEZ-GALINDO, M.D.**

Executive Vice-President, St. Jude Children's Research Hospital

Member and Chair, Global Paediatric Medicine, St. Jude Children's Research Hospital

Dr Carlos RODRIGUEZ-GALINDO is Executive Vice President and Chair of Global Paediatric Medicine at St. Jude Children's Research Hospital. A leader in paediatric oncology, he served as President of the Histiocyte Society and is an elected member of the Society for Paediatric Research and American Paediatric Society. He holds editorial roles in leading journals and reviews grants for major foundations. Dedicated to education, he received the Holcombe E. Grier Award for Excellence in Teaching at Harvard. His global efforts and academic leadership continue to shape paediatric cancer care and research worldwide.

**Rupert HANDGRETINGER, Prof. Dr. med**

George and Jennifer Yeo Endowed Chair of Paediatric Oncology, Yong Yeo Lin School of Medicine, National University of Singapore
Consultant, Paediatric Haematology/Oncology, Abu Dhabi Stem Cell Centre
Senior Professor, University of Tuebingen, Germany

Dr Rupert HANDGRETINGER is the George and Jennifer Yeo Endowed Chair of Paediatric Oncology at the National University of Singapore, Consultant at Abu Dhabi Stem Cell Center, and Senior Professor at the University of Tuebingen. A member of the German National Academy of Sciences Leopoldina, he has published over 550 peer-reviewed papers with an H-index of 99. He holds multiple patents in cancer immunotherapy and stem cell transplantation and received the PTCTC Lifetime Achievement Award. Dr. Handgretinger serves as Associate Editor for Bone Marrow Transplantation and reviews for top journals including NEJM, Nature Medicine, and Lancet.

**Julie Ruggieri PARK, M.D.**

Member, St. Jude Faculty
Chair, Department of Oncology
Associate Director Comprehensive Cancer Center, Translational Research
Endowed Chair in Paediatric Oncology

Dr Julie PARK is Chair of Oncology at St. Jude Children's Research Hospital and a leading paediatric hematologist-oncologist specializing in childhood cancer therapy. She has advanced neuroblastoma treatment through improved prognostic stratification and targeted therapeutics, serving as past Chair of the Children's Oncology Group Neuroblastoma Committee and current Scientific Chair of New Approaches to Neuroblastoma Therapy. Dr. Park has led first-in-human cellular immunotherapy trials, integrating tumour and immune profiling to enhance understanding of therapy response and toxicity. Her ongoing research focuses on uncovering molecular and immunologic vulnerabilities in paediatric cancers to guide the development of novel, more effective treatments.

**Ibrahim QADDOUMI, M.D., M.S.**

Director, Global Neuro-Oncology Program
Director, Jordan Program, St. Jude Global, St. Jude Children's Research Hospital

Dr Ibrahim QADDOUMI is Director of the Global Neuro-Oncology and Jordan Programs at St. Jude Children's Research Hospital, where he also serves as Associate Member in Oncology and Global Paediatric Medicine. He has pioneered treatment and research in low-grade gliomas and led international collaborations to establish sustainable neuro-oncology and ocular oncology services, notably at the King Hussein Cancer Center in Jordan. Dr. Qaddoumi has advanced telemedicine for global paediatric cancer care and launched the first Solid Tumors fellowship at St. Jude. His work continues to shape neuro-oncology education, research, and care across underserved regions worldwide.

**Melissa M. HUDSON, M.D**

Director, Cancer Survivorship Division
Member, Department of Oncology, St. Jude Children's Research Hospital

Dr Melissa Hudson is Director of the Cancer Survivorship Division at St. Jude Children's Research Hospital, where she has led the After Completion of Therapy (ACT) Clinic since 1993. Her work has shaped risk-based survivor care, integrating education, screening, and prevention for over 8,000 long-term childhood cancer survivors. She co-chaired the COG Long-Term Follow-Up Guidelines and the International Harmonization Group, and leads the St. Jude Lifetime Cohort Study. A widely published expert in survivorship outcomes, Dr. Hudson continues to advance care models and research that promote lifelong health for paediatric cancer survivors through coordinated, evidence-based strategies.

**Kevin KRULL, PhD**

Member and Chair
Department of Psychology and Biobehavioural Sciences
Endowed Chair in Psychology
St. Jude Children's Research Hospital

Dr Kevin Krull is Chair of Psychology and Biobehavioral Sciences and Endowed Chair in Psychology at St. Jude Children's Research Hospital. He leads the Neurocognitive Task Force of the International Guidelines Harmonization Group and serves multiple national and international committees focused on survivorship and late effects. A member of the St. Jude Graduate School and CCSS Executive Committee, Dr. Krull co-leads clinical trials including NEUROSTIM and SLEEPWELL. His research explores biologic mechanisms of neurocognitive impairment in paediatric leukemia survivors, aiming to develop targeted interventions that improve long-term cognitive outcomes and quality of life.

Keynote Abstracts

New Frontiers in the Genetics and Treatment of Childhood Acute Lymphoblastic Leukaemia

Ching-Hon Pui, St. Jude Children's Research Hospital

In recent years, comprehensive genomic and transcriptomic profiling has refined the classification of paediatric acute lymphoblastic leukemia (ALL), leading to the recognition of 23 B-ALL and 15 T-ALL genetic subtypes with important prognostic and therapeutic implications. For example, DUX4-rearranged ALL is now recognized as a distinct entity characterized by aberrant ERG expression and pronounced lineage plasticity, often exhibiting monocytic or myeloid features that contribute to treatment resistance and poor outcomes. Within the MEF2D-rearranged group, patients with MEF2D::BCL9 fusions generally have more favourable outcomes, whereas those with MEF2D::HNRNPUL1 fusions face higher relapse risk and inferior survival. In Philadelphia chromosome-positive (Ph+) ALL, some patients exhibit persistence of BCR::ABL1 transcripts by PCR despite achieving MRD negativity by flow cytometry; the long-term prognostic significance of this discordance remains uncertain because of limited follow-up. Research on genetic ancestry has shown that ancestral background influences both disease susceptibility and treatment outcomes. Several novel agents have demonstrated promising activity in childhood ALL. For B-cell ALL, bispecific T-cell engagers such as blinatumomab and antibody-drug conjugates like inotuzumab ozogamicin have significantly improved outcomes in both newly diagnosed and relapsed or refractory disease. CAR-T cell therapies targeting CD19 and CD22, including tisagenlecleucel and dual-target constructs, have achieved durable remissions in heavily pretreated patients. For T-cell ALL, emerging strategies include venetoclax for early T-cell precursor (ETP) or near-ETP ALL, dasatinib for more mature subtypes, and early-phase trials of CD7-directed CAR-T cells, all showing encouraging efficacy in resistant disease. Lineage switch from lymphoid to myeloid phenotypes has been increasingly recognized following CD19- or CD22-directed therapies, including antibodies and CAR-T cells. This phenomenon is most often seen in subtypes with inherent lineage plasticity, such as KMT2A-rearranged and DUX4-rearranged ALL. Finally, germline predisposition is now appreciated as an important contributor to paediatric ALL. Mutations in genes such as TP53, PAX5, and ETV6 not only drive leukemogenesis but also have implications for treatment decisions, long-term surveillance, and family counseling. Incorporating germline testing into clinical care supports precision medicine, facilitates risk-adapted therapy, and informs survivorship planning.

New Insights into the Biology, Diagnosis and Management of T-cell Acute Lymphoblastic Leukaemia and Lymphoma

David T. Teachey, The Children's Hospital of Philadelphia

Historically, outcomes for children, adolescents and young adults with T-cell acute lymphoblastic leukaemia (T-ALL) and lymphoblastic lymphoma (T-LL) were inferior to those with B-ALL/B-LL. Nevertheless, with modern therapy survival rates for those with T-ALL/T-LL and B-ALL/B-LL are similar, as cure rates for newly diagnosed patients with T-ALL/T-LL exceed 80%. While outcomes for de novo patients with T-ALL/T-LL have improved, survival for patients with relapsed disease remain poor, with overall survival rates of less than 30%. Accordingly, the primary goal in the treatment of patients with T-ALL/T-LL is to prevent relapse. Most of the improvements in cure rates have occurred through the intensification of cytotoxic chemotherapy regimens. Yet, a threshold has been reached where further intensification of chemotherapy has not improved survival. Potential mechanisms to improve cure rates include refining risk-stratification and integrating targeted therapies and immunotherapies into multiagent chemotherapy backbones.

Unlike B-ALL where recurrent somatic genomic alterations are used in risk-stratification, no alterations have been identified in T-ALL that are reproducibly prognostic independent of treatment response. Recent large scale genomic profiling efforts have demonstrated that >60% of leukaemia drivers in T-ALL are caused by alterations in non-coding or intragenic regions in the genome. Many of these genomic alterations can be missed by traditional exon-based sequencing approaches, requiring whole genome sequencing (WGS) for identification. Further, a number of these genomic alterations may be prognostic independent of treatment response.

The use of WGS and transcriptome profiling (RNAseq) may allow for the identification of patients with low-risk of relapse who may benefit from deintensification of therapy, as well as patients with high-risk of relapse who may benefit from alternative therapies or stem cell transplant.

Several targeted therapies have been identified with promising data in preclinical T-ALL models, including inhibitors of BCL2/BCLXL (venetoclax, navitoclax), CDK4/6 (palbociclib, ribociclib), Jak/Stat (ruxolitinib, tofacitinib), LCK/SRC (dasatinib, ponatinib), menin (revumenib, ziftomenib), and the proteasome (bortezomib, carfilzomib). While some of these agents have shown efficacy in early phase clinical trials, the percentage of patients who may benefit from many of these drugs is <50%, as the genomic landscape of T-ALL is quite heterogeneous. Hopefully, enhanced genomic profiling of T-ALL patient blasts will identify biomarkers that can be used in the clinic to identify patients most likely to benefit from different targeted agents. Several novel immunotherapies have also shown very promising activity in preclinical models and early phase trials, including monoclonal antibodies targeting CD38 (daratumumab) and chimeric antigen receptor T-cells (CAR-T) targeting CD7 and CD5.

Keynote Abstracts

Finally, while the treatment of patients with T-ALL and T-LL has harmonized over time, recent trials have shown differential benefit to novel agents. Patients with T-ALL but not T-LL benefited from nelarabine on the Children's Oncology Group (COG) AALL0434 clinical trial and patients with T-LL but not T-ALL benefited from bortezomib on the COG AALL1231 clinical trial, highlighting the need to understand biologic and microenvironmental differences between T-ALL and T-LL.

This talk will discuss recent data on risk stratification, targeted therapies and novel immunotherapies for children and young adults with T-ALL and T-LL.

T-cell Immunity and Its Impact on ALL Treatment Response

Jun YANG, St. Jude Children's Research Hospital

Although cure rates of ALL improve significantly over the past 5 decades, not all children benefited equally from this impressive progress, with a substantial proportion of patients experiencing relapse and/or debilitating toxicities of ALL therapy. This inter-patient variability is often attributed to leukaemia genomics, ALL drug sensitivity, germline genetic variants associated with drug metabolism and disposition. By contrast, the role of host immunity, either innate or adaptive, in long-term cure of ALL remains poorly understood. In a proof-of-principle study using syngeneic mouse model, we demonstrated that loss of T cells in the host drastically increased leukaemia relapse after targeted or cytotoxic chemotherapy. In particular, T-cell immunity was essential for suppressing the outgrowth of drug-resistant leukaemia and T cell activation can improve antileukemia efficacy of chemotherapy *in vivo*. T-cell abundance in patients with ALL also exhibited a significant association with treatment outcomes. To further characterize this, we recently conducted a comprehensive study to examine the pattern and dynamic change of T cell diversity in children with ALL, as a proxy marker for host immunity. We evaluated TCR repertoire for its association with ALL molecular subtypes, early response, and long-term survival. Together, these results indicate that T-cell immunity plays pivotal roles in maintaining long-term remission of ALL, highlighting that the interplay between host immunity and drug response can be harnessed to further individualize ALL therapy.

CAR T Cell Therapy in Paediatric Oncology: Current Status and Future Prospects

Crystal MACKALL, Center for Cancer Cell Therapy, Stanford Cancer Institute

Immunotherapy for cancer has transformed the treatment of adult cancer. In paediatric cancer, outcomes for B-ALL, the most common paediatric cancer, have steadily improved over the last five decades, most recently due to impressive activity of chimeric antigen receptor modified T (CAR-T) cells and bispecific antibodies targeting CD19. These results provide proof-of-concept that immunotherapy in general, and chimeric antigen receptor modified T cells in particular, have promise for improving outcomes for paediatric cancers. Significant effort is underway to expand the reach of CAR T cell therapy in paediatric cancer beyond B-ALL and promising clinical activity using CAR modified T cells has been demonstrated in several additional paediatric cancers, including GD2-CAR T cells for neuroblastoma and diffuse midline glioma, CD30-CAR T cells for Hodgkin's disease, CD5- or CD7-CAR T cells for T-ALL and CD22-CAR T cells for B-ALL. Brain tumors currently kill more children in the developed world than any other cancer and novel therapeutics are needed for these tumours. This presentation will discuss emerging data using GD2-CAR T cells to treat diffuse midline gliomas, and preclinical work in developing GPC2-CAR T cells for medulloblastoma. GD2 is massively overexpressed by diffuse midline glioma as a direct result of the H3K27M oncogene, which prevents silencing of the major enzyme responsible for regulating GD2 synthesis. Our team at Stanford developed a GD2-CAR that mediates potent activity against diffuse midline gliomas in preclinical models. We subsequently translated this to the clinic and have treated more than 40 patients with intravenous and/or intracerebroventricular GD2-CAR T cells. Lessons from this experience will be presented, including safety outcomes, efficacy outcomes and emerging understanding regarding mechanisms of resistance. Medulloblastoma is the most common brain tumour of childhood. We discovered significant overexpression of glycan 2 in medulloblastoma tumors and we utilized preclinical models to develop and optimize a GPC2-CAR with significant activity in preclinical models of medulloblastoma. We have recently opened a first-in-human, first-in-child Phase I trial of GPC2-CAR for medulloblastoma.

Curing Children with Cancer: Strategies to Address Global Disparities in Childhood Cancer Care and Control

Carlos Rodriguez-Galindo, St. Jude Children's Research Hospital

Cure rates for childhood cancer are now above 80% with modern therapies and supportive care. However, each year, close to 400,000 children develop cancer worldwide, most of them in countries with limited resources, where access to diagnosis, essential medicines, or adequate care. While cure rates exceed 80% in high-income countries, fewer than 20% of children in low- and middle-income countries (LMICs) survive. Avoidable deaths result from lack of diagnosis, misdiagnosis or delayed diagnosis, obstacles to accessing care, abandonment of treatment, death from toxicity and relapse. And yet, sustainable and cost-effective models are possible.

Keynote Abstracts

Several global initiatives have been developed to address these disparities. In 2018, WHO launched the Global Initiative for Childhood Cancer (GICC), which aims to achieve 60% survival for children with cancer globally by 2030. The GICC works with governments to develop national strategies to strengthen the health systems and design national childhood cancer control plans. In parallel, St. Jude Global, through its Department of Global Paediatric Medicine and the St. Jude Global Alliance of over 400 institutions in 93 countries, aims to accelerate those aims through a systemic, multi-level engagement—from hospitals to national health systems—emphasizing partner empowerment, capacity building, education, research, and advocacy. Key collaborations include designations as a WHO Collaborating Centre for Childhood Cancer and partnerships with UNICEF, PAHO, IAEA, and others to strengthen diagnostics, treatment, and health policies. Flagship initiatives include the Global Platform for Access to Childhood Cancer Medicines developed in partnership with WHO, UNICEF, and PAHO and that aims to provide uninterrupted supply of quality essential cancer medicines to children with cancer in LMICs. Complementing these efforts, St. Jude Global integrates research methodology in quality improvement, implementation science, epidemiology, and health economics to generate evidence and guide policy.

Advances in Haploidentical Transplantation

Rupert HANDGRETINGER, Children's University Hospital Tuebingen

Over the last decade, the numbers of haploidentical transplants have increased worldwide. The clinical outcome has significantly improved and is almost reaching the outcomes of matched unrelated and also matched sibling transplants. There are a number of potential advantages of haploidentical transplantation: The rapid availability of a donor in the absence of matched siblings, the availability of the donor for post-transplant immunotherapeutic strategies, such as donor lymphocyte infusions (DLI), CD45-RA depleted T-cell infusions, Natural killer (NK) or gamma/delta T-cell infusions or even donor-derived CAR T cell therapies. Another potential advantage is the disparity in HLA class I antigens, which allows a rapid and sensitive analysis of the chimerism status of the patient and also might allow the early detection of impending relapses in malignant diseases. There are still a number of issues which can be improved, such as the tolerization of donor T-cells by post-transplant cyclophosphamide to prevent Graft-versus-Host Disease (GvHD) or the tolerization of recipient T-cells which were not eliminated by the conditioning regimen and can lead to rejection of the graft. Several new approaches to improve the outcome of haploidentical transplantation will be discussed.

Recent Advances in Treatment of High-Risk Neuroblastoma

Julie PARK, St. Jude Children's Research Hospital

High-risk neuroblastoma remains a formidable clinical challenge, with the majority of patients ultimately succumbing to the disease despite aggressive multimodal therapies — including chemotherapy intensification, differentiation therapy, and immunotherapy. Survival for children who relapse during or after standard treatment remains dismally low, under 10%, and current regimens relying on dose-intensive cytotoxic agents and radiation are associated with significant long-term toxicities, underscoring the urgent need for more effective and less harmful therapeutic strategies. This lecture will provide a comprehensive overview of recent breakthroughs in the management of high-risk neuroblastoma, including the emergence of molecularly targeted therapies for genetically defined patient subsets, advances in targeted radiotherapeutics such as I-131-MIBG, and the expanding role of immunotherapies, notably anti-GD2 monoclonal antibodies and cellular therapies. Special emphasis will be placed on innovative approaches currently in clinical development that aim to enhance the efficacy of immunotherapy for children with relapsed and refractory disease.

Personalized and Tailored Approach for Paediatric Low Grade Gliomas

Dr Ibrahim QADDOUMI, St. Jude Children's Research Hospital

Paediatric Low Grade Gliomas (LGG) are diverse group of tumours that vary in their presentation, pathology, clinical behaviour, and molecular signature. With new discoveries in genetic alterations in BRAF, NF1, mTOR, FGFR, IDH1/2 and others it is natural to consider personalized approach by using alteration-specific targeted therapy. However, traditional tailored approach through early diagnosis, surgical precision and most important of all team approach are still equally important therapies and should not be abandoned for expensive new targeted therapies.

Keynote Abstracts

Supporting Adherence to Childhood Cancer Survivorship Care

Melissa HUDSON, St. Jude Children's Research Hospital

Progress in biology and therapy for paediatric cancers has produced a growing population of long-term (exceeding 5 years) cancer survivors who are at increased risk for morbidity and premature mortality related directly to the cancer itself, to pre- and co-existing comorbidities, and to exposure to cancer treatment modalities. Consequently, cancer survivors represent an important group that may benefit from risk assessment, disease prevention services, and health promotion counselling. Risk-based survivor care that includes tailored screening, surveillance, and prevention based on the previous cancer, cancer therapy, genetic predispositions, lifestyle behaviours, and co-morbid health conditions is recommended for all survivors. To optimize risk-based survivor care, several groups have organized health screening guidelines based on evidence from the literature linking specific therapeutic interventions with late treatment complications.

To facilitate care coordination among oncologists and community providers, the use of a written treatment summary and care plan is recommended to communicate the survivor's health status, provide a care roadmap to ensure survivor-appropriate services, and clearly delineate provider roles. However, adherence to this recommendation by oncology providers remains suboptimal because of the significant time and resource barriers involved in organizing survivorship care plans. Identification of the essential components of survivorship care plans, which may vary across health care settings, is important to facilitate their widespread adoption. The integration of automated, programmable applications within existing electronic health record systems may expedite the development of care plan summaries in the future. To enhance awareness of survivorship health issues, educational efforts must be expanded to target not only oncology providers, but also practicing clinicians, graduate medical trainees, and survivors.

Continued follow-up during adulthood is essential to accurately characterize very late cancer-related sequelae and determine if complications resulting from cancer therapy will be exacerbated by the organ dysfunction associated with aging. In this way, late health outcomes research plays a critical role in refining screening/surveillance recommendations and guiding the development of preventive and remedial interventions to preserve health.

This presentation will review the scope of long-term health effects after paediatric cancer and the challenges in coordinating long-term survivor care and discuss strategies and resources to support adherence to the delivery of risk-based survivorship care.

Biologic Mechanisms Associated with Neurocognitive Impairment in Survivors of Paediatric Acute Lymphoblastic Leukaemia

Kevin KRULL, St. Jude Children's Research Hospital

Roughly 40% of long-term survivors of paediatric leukaemia treated on contemporary chemotherapy-only protocols experience neurocognitive impairment. This impairment begins by the end of therapy and progresses overtime, negatively impacting future educational and vocational attainment, as well as quality of life. Recent evidence suggests increased risk for early onset cognitive decline and, potentially, dementia. This presentation will review evidence of onset and progression of neurocognitive decline and discuss multiple central nervous system and peripheral physiological changes that are associated with this progression, some of which begin within the first months following diagnosis. Interventions that target the altered biological mechanisms will also be reviewed.



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Abbreviations: GD2, disialoganglioside.

References: 1. HK-Package Insert: SPC-DIN-HK-1171-06

Date of preparation: October 2025.

This material has been developed by Recordati Rare Diseases & FarmaMondo.

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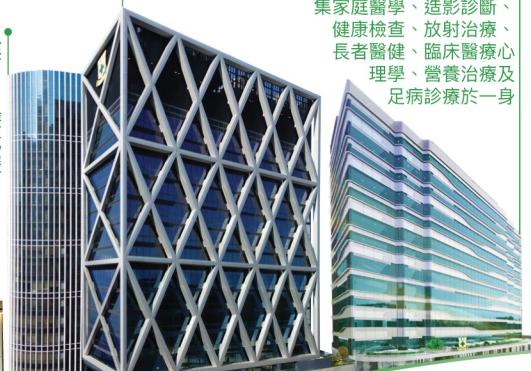
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1. Data on file. Available from CSL Behring as DOF-PRI-10019
2. Data on file. Available from CSL Behring as DOF-PRI-10020
3. Bölli R, Woodill K, Bartschi M, et al. L-proline reduces IgG dimer content and enhances the stability of intravenous immunoglobulin (IVIG) solutions. *Biologicals*. 2010;38(1):150-157.
4. Hong Kong Privigen Package Insert, Nov 2021

Ig: immunoglobulin; IVIG: intravenous immunoglobulin; bw: body weight; PID: Primary immunodeficiency syndromes; SID: Secondary immunodeficiencies; CIDP: Chronic inflammatory demyelinating polyneuropathy; ITP: Primary immune thrombocytopenia; GBS: Guillain-Barré syndrome; MMN: Multifocal Motor Neuropathy

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The Pau Kwong Wun Charitable Foundation (PKWCF) was established in 1998 by Benson and Ammy Pau in memory of their son. The foundation was created to extend the love the couple devoted to him during the final stage of his battle with leukemia, before his passing at the age of ten. In 2004, PKWCF became a member of Childhood Cancer International (CCI), broadening its international perspective and actively engaging in global childhood cancer initiatives.



世界衛生組織「全球兒童癌症倡議」
(WHO Global Initiative for Childhood Cancer)

GICC
目標
Target

- 2030年，全球兒童癌症治愈率至少達到60%
- 減少所有患者及其家庭的痛苦
- survival rate of children with cancer globally to at least 60% by 2030
- reducing their suffering and improving their quality of life

PKWCF
回應
Response

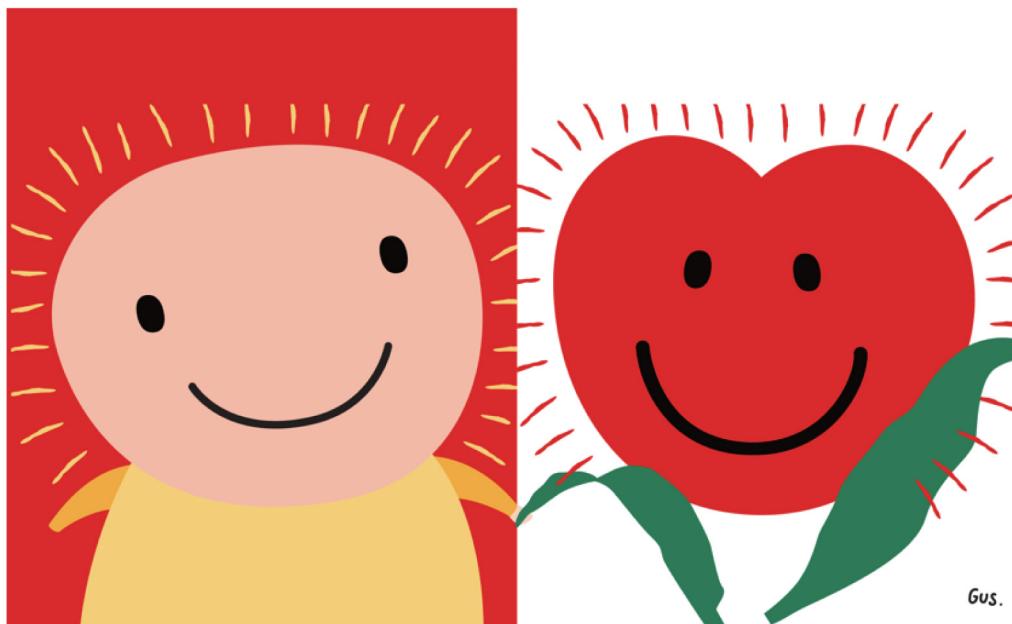
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In alignment with the WHO GICC, the foundation began promoting and funding a childhood cancer projects in 2024. These efforts focus on three key areas: Training healthcare professionals to enhance regional medical capacity; promoting early screening, diagnosis, and treatment; supporting service delivery and capacity building for non-profit organizations.

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