

Discovery and Innovation in Pediatric Cancer- From Biology to Breakthrough Therapies

In association with the AACR Pediatric Cancer Working Group (PCWG)
September 25-28, 2025 | The Westin Copley Place | Boston, MA

Conference Cochairs:

Alanna J. Church, Boston Children's Hospital, Boston, MA

Meredith S. Irwin, SickKids, Toronto, ON, Canada

Charles W.M. Roberts, St. Jude Children's Research Hospital, Memphis, TN

Thursday, September 25, 2025

2:00	Registration <i>America Foyer</i>
5:00-5:05	Welcome
5:05- 5:10	Introduction
5:10-5:50	Keynote: Oncohistones and the 3D Chromatin Code of Cancer Nada Jabado, McGill University, Montreal, Canada <i>America North & Center</i>
5:50-5:55	Introduction
5:55-6:35	Curing the curable: towards global precision medicine in childhood ALL AACR-St. Baldrick's Foundation Pediatric Cancer Research Fellowship Awardee Lecture Shawn HR Lee, National University of Singapore, Singapore <i>America North & Center</i>
6:35- 6:55	Discussion & QA Panel
7:00-9:30	Poster Session A & Opening Reception <i>America South</i>

Friday, September 26, 2025

Continental Breakfast

7:00-8:00 a.m. |

America Foyer

Plenary Session 1: Mechanisms underlying childhood cancer

8:00-10:00 a.m.

America North & Center

Session Chair: Liling Wan, Perelman school of Medicine, Philadelphia, PA

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- 8:00-8:20 **Chromatin in cancer: from mechanisms to emerging therapies**
Charles Roberts, St. Jude Children's Research Hospital, Memphis, TN
- 8:20-8:30 Discussion/Q&A
- 8:30-8:50 **Targeting intra-tumor heterogeneity and developmental targets in pediatric brain tumors**
Mariella G. Filbin, Dana-Farber Cancer Institute, Boston, MA
- 8:50-9:00 Discussion/Q&A
- 9:00-9:20 **Chromatin-Associated Condensates as Drivers of Pediatric Cancer**
Liling Wan, Perelman school of Medicine, Philadelphia, PA
- 9:20- 9:30 Discussion/Q&A
- 9:30-9:45 **CDK8 Inhibition Releases the Muscle Differentiation Block in Fusion-Driven Alveolar RhabdomyosarcomaS***
Susu Zhang, Dana-Farber Cancer Institute, Boston, MA
- 9:45- 10:00 **Membrane localization mediated by BRAF fusion partners is necessary for transformation of human neural stem cells***
Alexander Zhang, Dana Farber Cancer Institute, Boston, MA

Break

10:00-10:30 a.m. |

Plenary Session 2: Development in cancer

10:30 a.m.-12:30 p.m. |

America North & Center

Session Chair: Pratiti (Mimi) Bandopadhyay, Dana-Farber Cancer Institute, Boston, MA

- 10:30-10:50 **The interplay between brain development and pediatric brain tumors**
Pratiti (Mimi) Bandopadhyay, Dana-Farber Cancer Institute, Boston, MA
- 10:50-11:00 Discussion/Q&A
- 11:00-11:20 **Dissecting cell fate transitions in the malignant brain tumour hierarchy**
Peter B. Dirks, SickKids, Toronto, ON, Canada
- 11:20-11:30 Discussion/Q&A
- 11:30-11:50 **Targeting (and using) enhancers to control cell state in pediatric cancers**
Adam D. Durbin, St. Jude Children's Research Hospital, Memphis, TN
- 11:50-12:00 Discussion/Q&A

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12:00-12:15 **EWS::FLI1 Expression in Human Embryonic Mesenchymal Stem Cells Leads to Transcriptional Reprogramming, defective DNA Damage Repair and Ewing Sarcoma.***
Jaume Mora, Pediatric Cancer Center Barcelona, Barcelona, Spain

12:15- 12:30 **KAT6A/B are therapeutic targets to enhance the efficacy of differentiation therapy and GD2 immunotherapy in neuroblastoma***
Nina Weichert-Leahey, Dana-Farber Cancer Institute, Boston, MA

Lunch Break (lunch on your own)

12:30-2:30 p.m.

Plenary Session 3: From targets to trials: Rethinking how we design child-first medicine

Organized by the CRUK

2:30-5:00 p.m.

America North & Center

Session Chair: David Jenkinson, LifeArc, London, United Kingdom

2:30- 2:35 p.m. **Introduction**
David Jenkinson, LifeArc, London, United Kingdom

2:35- 2:50 p.m. **Cayden's Courage: A Journey Through Childhood Cancer**
Courtney Addison

2:50- 3:50 p.m. **Scientific Presentations:**
Navigating the landscape of paediatric oncology modalities

Epigenetic Dependence of Pediatric Brain Tumor - Insight into Epigenetic Therapy
Rintaro Hashizume, University of Alabama at Birmingham, Birmingham, AL

Targeted protein degradation for the treatment of pediatric malignancies
Benjamin L. Ebert, Dana-Farber Cancer Institute, Boston, MA

Precision Radiotherapy for Incurable Brain Tumors: Phase 1b Dose & Regimen Optimization Study of Iopofosine I 131 in Inoperable Relapsed or Refractory Pediatric High- Grade Glioma, Interim Data Assessment
Jarrod Longcor, Collectar Biosciences, Florham Park, NJ

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- 3:50- 4:45 p.m. **Panel Discussion: Rewriting the rules of childhood cancer translation**
Panelists:
Catherine Bollard, Children's National, Washington, DC
Benjamin L. Ebert, Dana-Farber Cancer Institute, Boston, MA
Mariella G. Filbin, Dana-Farber Cancer Institute, Boston, MA
Rintaro Hashizume, University of Alabama at Birmingham, Birmingham, AL
Charles Roberts, St. Jude Children's Research Hospital, Memphis, TN
Jarrod Longcor, CellerBio, Inc., Florham Park, NJ
- 4:45- 5:00 **A year of C-Further**
Tony Hickson, Cancer Research Horizon

Poster Session B (with light refreshments)

5:00- 7:00 p.m. |

Saturday, September 27, 2025

Continental Breakfast

7:00-8:00 a.m. |

America Foyer

Plenary Session 4: Discovery and translation of targets

8:00-10:00 a.m. |

America North & Center

Session Chair: Kimberly Stegmaier, Dana-Farber Cancer Institute, Boston, MA

- 8:00-8:20 **Therapeutic vulnerabilities in NUP98-rearranged leukemia**
Charles Mullighan, St. Jude Children's Research Hospital, Memphis, TN
- 8:20-8:30 Discussion/Q&A
- 8:30-8:50 **New Approaches to Target Pediatric Cancers**
Kimberly Stegmaier, Dana-Farber Cancer Institute, Boston, MA
- 8:50-9:00 Discussion/Q&A
- 9:00-9:20 **Targeting chromatin modulators with therapeutic degraders**
Gromek Smolen, Foghorn Therapeutics, Cambridge, MA

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9:20-9:30 Discussion/Q&A

9:30- 9:45 **The Potential of BCL-xL degradation as a strategy to eliminate neuroblastoma persister cells***
Matteo Calafatti, Children's Hospital of Philadelphia, Philadelphia, PA

9:45- 10:00 **CBL mutations in pediatric solid and CNS tumours***
Paul Ekert, Children's Cancer Institute, Sydney, NSW, Australia

Break

10:00-10:30 a.m. |

Plenary Session 5: Immunology and Immuno-oncology in Pediatrics

10:30 a.m.-12:45 p.m. |

America North & Center

Session Chair: Catherine Bollard, Children's National, Washington, DC

10:30-10:50 **Next Generation T cell Therapies for Pediatric Solid Tumors**
Catherine Bollard, Children's National, Washington, DC

10:50-11:00 Discussion/Q&A

11:00-11:20 **A portfolio of cellular immunotherapies for ALK-driven cancers**
Roberto Chiarle, Boston Children's Hospital, Brookline, MA

11:20-11:30 Discussion/Q&A

11:30-11:50 **GD2-CAR T01 cells for pediatric solid tumors**
Francesca Del Bufalo, Bambino Gesù Children's Hospital, Rome, Italy

11:50-12:00 Discussion/Q&A

12:00-12:15 **Characterization of Cytotoxic Cell Abundance and Genetic Variants in Immune-related Genes in Pediatric Acute Lymphoblastic Leukemia Patients***

Maria Sol Ruiz, Universidad de Buenos Aires, Buenos Aires, Argentina

12:15- 12:30 **Alectinib in Children and Adolescents with Solid or CNS Tumors Harboring ALK-fusions: Updated Data from the iMatrix Alectinib Phase I/II Open-label, Multi-center Study***

Francis Mussai, Roche, Welwyn Garden City, United Kingdom

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Lunch Break (lunch on your own)

12:30-3:00 p.m.

Plenary Session 6: Cancer biology, early detection and ctDNA

3:00- 5:00 p.m. |

America North & Center

Session Chair: Paul Northcott, St. Jude Children's Research Hospital, Memphis, TN

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| 3:00- 3:20 | DDR deficiency in neuroblastoma: lessons from zebrafish and patients
Meredith Irwin, SickKids, Toronto, ON, Canada |
| 3:20-3:30 | Discussion/Q&A |
| 3:30-3:50 | Leveraging liquid biopsy to transform patient care in pediatric neuro-oncology
Paul Northcott, St. Jude Children's Research Hospital, Memphis, TN |
| 3:50-4:00 | Discussion/Q&A |
| 4:00-4:20 | Cell-free DNA sequencing for early detection of hereditary cancer
Trevor Pugh, UHN Princess Margaret Cancer Centre, Toronto, ON, Canada |
| 4:20-4:30 | Discussion/Q&A |
| 4:30-4:45 | Whole-Genome-Based, Tumor-Informed Circulating Tumor DNA Detection Correlates With Treatment Response And Survival Of Pediatric Solid Tumor*
Loretta Lau, Children's Cancer Institute, Sydney, Australia |
| 4:45- 5:00 | Implementation and Performance of a Neuroblastoma Liquid Biopsy Panel for Non-Invasive Molecular Monitoring*
Lea Surrey, Children's Hospital of Philadelphia, Philadelphia, PA |

Sunday, September 28, 2025

Continental Breakfast

7:00-8:00 a.m.

America Foyer

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Plenary Session 7: Molecular Detection and Diagnostics

8:00-10:00 a.m. |

America North & Center

Session Chair: Sam Behjati, Wellcome Sanger Institute, Cambridge, UK

8:00-8:20 **Building Consensus, Driving Coverage: Toward Universal Molecular Profiling in Pediatric Cancer**

Alana Church, Boston Children's Hospital, Boston, MA

8:20-8:30 Discussion/Q&A

8:30-8:50 **How Whole Genome Sequencing Helps Children with Cancer**

Sam Behjati, Wellcome Sanger Institute, Cambridge, UK

8:50-9:00 Discussion/Q&A

9:00-9:20 **Enabling precision oncology in pediatric soft tissue sarcoma**

Jack Shern, National Cancer Institute, Bethesda, MD

9:20-9:30 Discussion/Q&A

9:30- 9:45 **Precision Diagnosis in High-Risk Paediatric Oncology through Integrated Whole Genome, Transcriptome, and Epigenome Profiling***

Mark Cowley, Children's Cancer Institute, Sydney, Australia

9:45- 10:00 **Investigating the potential of extracellular vesicle biomarkers for synovial sarcoma detection***

Maryke Grobler, Treehouse Childhood Cancer Initiative, Santa Cruz, CA

Break

10:00-10:30 a.m. |

Plenary Session 8: Clinical Trials

10:30 a.m.-12:15 p.m. |

America North & Center

Session Chair: Julie Park, St. Jude Children's Research Hospital, Memphis, TN

10:30-10:50 **Challenges in Accessing Innovation for Children with Cancer**

Steve DuBois, Dana-Farber Cancer Institute, Boston, MA

10:50-11:00 Discussion/Q&A

11:00-11:20 **Moving the Needle to Cure Every Children with ALL—What Will it take?**

Mignon Loh, Seattle Children's Research Institute, Seattle, WA

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11:20-11:30	Discussion/Q&A
11:30-11:50	Immunotherapy: Shifting the Paradigm for the Treatment of Solid Tumors In Pediatric and Young Adults Patients Julie Park, St. Jude Children's Research Hospital, Memphis, TN
11:50-12:00	Discussion/Q&A
12:00-12:15	B7-H3-CAR T cells expressing 41BB ligand for pediatric solid tumors: Preliminary results of a phase 1 study* Rebecca Epperly, St. Jude Children's Research Hospital, Memphis, TN

Closing Remarks

12:15-12:30 p.m. |

*- short talks from highly rated abstracts