CONFERENCE PROGRAM

Tuesday, September 17

5:00 p.m.–6:30 p.m. **OPENING PLENARY SESSION**
Grand Salon Opera AB

Pediatric neuro-oncology: What's next?
Stefan M. Pfister, Hopp Children's Cancer Center at the NCT Heidelberg (KiTZ) and German Cancer Research Center, Heidelberg, Germany

Three-hit model of Wilms tumor formation reveals immunogenic transcriptional subtypes*
Kenneth Chen, UT Southwestern, Dallas, TX

Generation of the first genetically defined tumorigenic model of Ewing Sarcoma expressing EWS-FLI1*
Nilay Shah, Nationwide Children's Hospital, Columbus, OH

6:30 p.m.–8:00 p.m. **OPENING RECEPTION**
Grand Salon Opera Foyer

Wednesday, September 18

7:00 a.m.–8:00 a.m. **BREAKFAST AND NETWORKING ROUNDTABLES**
Grand Salon Opera C

8:00 a.m.–10:15 a.m. **PLENARY SESSION 1: CANCER PREDISPOSITION AND SURVEILLANCE**
Grand Salon Opera AB

Session Chair: David Malkin, The Hospital for Sick Children, Toronto, ON, Canada

Sex ratio disparities and the risk of childhood cancer: Evaluating the mediating effect of birth defects among 15,000 childhood cancer cases*
Erin Marcotte, University of Minnesota, Minneapolis, MN

Novel strategies for early cancer detection and prevention: The Li-Fraumeni Syndrome story
David Malkin

Medulloblastoma predisposition according to molecular subgroup: The usual suspects and beyond
Paul A. Northcott, St. Jude Children's Research Hospital, Memphis, TN

New approaches to study cancer predisposition syndromes
Christian P. Kratz, Hannover Medical School, Hannover, Germany

Circulating tumor DNA as a tool for prognostication, translational discovery, and early cancer detection
Brian Crompton, Dana-Farber Cancer Institute, Boston, MA

**Short talk from proffered abstract*

New strategies for multi-dimensional molecular characterization and follow-up of high-risk pediatric cancers
Gudrun Schleiermacher, Institut Curie, Paris, France

10:15 a.m.–10:30 a.m. **BREAK**
Grand Salon Opera Foyer

10:30 a.m.–12:30 p.m. **PLENARY SESSION 2: IMMUNOTHERAPY I: CELLULAR THERAPIES**
Grand Salon Opera AB

Session Chair: Crystal L. Mackall, Stanford University School of Medicine, Stanford, CA

Next-generation CAR T cells to overcome resistance
Crystal L. Mackall

Improving remission durability after CAR T cell therapy
Terry J. Fry, University of Colorado Denver, Aurora, CO

Immunotherapeutic approaches for pediatric solid tumors
Rupert Handgretinger, University of Tübingen, Tübingen, Germany

Locoregionally administered B7H3-targeting CAR T cells mediate potent antitumor effects in atypical teratoid/rhabdoid tumor*
Johanna Theruvath, Stanford University, Palo Alto, CA

T-cell receptor (TCR)-based immunotherapy in pediatric malignancy: Addressing the challenge of early metastasis and low immunogenicity*
Stefan Burdach, Department of Pediatrics and Children's Cancer Research Center, Technical University of Munich School of Medicine and CCC München - Comprehensive Cancer Center, DKTK German Cancer Consortium, Munich, Bavaria, Germany

12:30 p.m.–2:30 p.m. **LUNCH ON OWN**

2:30 p.m.–4:30 p.m. **PLENARY SESSION 3: IMMUNOTHERAPY II: IMMUNE CHECKPOINT INHIBITION AND TUMOR MICROENVIRONMENT**
Grand Salon Opera AB

Session Chair: Paul Sondel, University of Wisconsin School of Medicine, Madison, WI

Activating innate and adaptive immunity to improve outcome for "cold" tumors
Paul Sondel

Overcoming immune evasion in pediatric brain tumors
Robert J. Wechsler-Reya, Sanford Burnham Prebys Medical Discovery Institute, La Jolla, CA

Rebalancing the immune milieu in the early metastatic microenvironment
Rosandra N. Kaplan, National Cancer Institute, Bethesda, MD

The immunogenomic landscape of pediatric primary solid tumors*
Arash Nabbi, Princess Margaret Cancer Centre, University Health Network, Toronto, ON, Canada

**Short talk from proffered abstract*

GD2 is a macrophage checkpoint molecule and combined GD2/CD47 blockade results in synergistic effects and tumor clearance in xenograft models of neuroblastoma and osteosarcoma*
Robbie Majzner, Stanford University School of Medicine, Stanford, CA

4:30 p.m.–4:45 p.m. **SPECIAL SESSION 1: LIGHTNING LECTURES**
Grand Salon Opera AB

Session Chair: David Malkin, The Hospital for Sick Children, Toronto, ON, Canada

Lightning Lectures highlight the top research that will be presented as posters. Selected abstracts from Poster Session A will be featured in this session. Presenters will have 1 minute to present 1 slide to highlight their most exciting finding. We invite you to discuss these in greater depth during the poster session.

Prevalence and spectrum of germline mutations in children with high-risk cancer
Noemi Fuentes-Bolanos, Kid's Cancer Center, Sydney, NSW, Australia

A comprehensive and integrative omic analysis of multiply relapsed refractory pediatric pre-B cell acute lymphoblastic leukemia predicts response to CD19 CAR T cell therapy
Katherine Masih, National Institutes of Health, Bethesda, MD

Glypican-2 targeted CAR T-cells designed to effectively eradicate endogenous site density solid tumors in the absence of toxicity
Neder Heitzeneder, Stanford Cancer Institute, Stanford, CA

Development of FGFR4 specific chimeric antibody receptor (CAR) T cell and bispecific T cell engager (BiTE) for rhabdomyosarcoma (RMS) immunotherapy
Adam Cheuk, National Cancer Institute, Bethesda, MD

Targeted sequencing in 388 patients with high-risk or recurrent/refractory pediatric extracranial solid malignancies: An interim report from the GAIN Consortium/iCat2 Study*
Laura Corson, Dana-Farber Cancer Institute, Boston, MA

BMI1 constitutes a novel therapeutic vulnerability in fusion-positive rhabdomyosarcoma
Robert Schnepp, Aflac Cancer and Blood Disorders Center, Department of Pediatrics, Division of Pediatric Hematology, Oncology, and Bone Marrow Transplant, Emory University School of Medicine, Atlanta, GA

Charting the synthetic lethality landscape in pediatric cancer to advance whole-exome precision-based treatments
Fiorella Schischlik, National Cancer Institute, Bethesda, MD

Defining the transcriptional regulation of pediatric AML as a new strategy to find potential druggable vulnerabilities*
Joanna Yi, Baylor College of Medicine, Houston, TX

EphB2 a potential therapeutic target for pediatric medulloblastoma
Yuchen Li, QIMR Berghofer Medical Research Institute, Brisbane, QLD, Australia

Zero Childhood Cancer (ZERO): A comprehensive precision medicine platform for children with high-risk cancer
Vanessa Tyrrell, Children's Cancer Institute, Sydney, NSW, Australia

**Short talk from proffered abstract*

Validation of potential therapies for treatment of fatal pediatric brain tumors DIPG and AT/RT using a novel rapid intracranial model in zebrafish
Harpreet Kaur, Johns Hopkins University, Baltimore, MD

Overcoming challenges in health care with machine learning: Innovation from retinoblastoma
Isabella Janusonis, The Hospital for Sick Children, Toronto, ON, Canada

4:45 p.m.–7:15 p.m. **POSTER SESSION A / LIGHT REFRESHMENTS**
Soprano Ballroom

Thursday, September 19

7:00 a.m.–8:00 a.m. **BREAKFAST AND NETWORKING ROUNDTABLES**
Grand Salon Opera C

8:00 a.m.–10:00 a.m. **PLENARY SESSION 4: MOLECULARLY TARGETED THERAPIES**
Grand Salon Opera AB

Session Chair: Gilles Vassal, Institut Gustave Roussy, Villejuif, France

Molecular targeted therapies and precision medicine for children with neuroblastoma and other refractory malignancies
Michelle Haber, Children's Cancer Institute, Randwick, NSW, Australia

Molecular profiling in the clinic: Moving from feasibility assessment to evaluating clinical impact
Katherine A. Janeway, Dana-Farber Cancer Institute, Boston, MA

Tissue-agnostic development of TRK inhibitors for pediatric cancer
Theodore Laetsch, UT Southwestern Medical Center, Dallas, TX

Accelerating innovation for children with cancer in the new regulatory environment
Gilles Vassal

10:00 a.m.–10:30 a.m. **BREAK**
Grand Salon Opera Foyer

10:30 a.m.–12:30 p.m. **PLENARY SESSION 5: PATHWAYS OF ONCOGENESIS I: 'OMICS**
Grand Salon Opera AB

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

From functional genomics to new targets in pediatric oncology
Kimberly Stegmaier

Clinically relevant mutational signatures in childhood cancer
Adam Shlien, The Hospital for Sick Children, Toronto, ON, Canada

Genomic analysis of osteosarcoma: Insights into tumor evolution and therapy response
E. Alejandro Sweet-Cordero, University of California San Francisco, San Francisco, CA

Neuronal activity promotes proliferation of normal and neoplastic glial cells
Michelle L. Monje, Stanford University School of Medicine, Stanford, CA

12:30 p.m.–12:50 p.m. **SPECIAL SESSION 2: LIGHTNING LECTURES**
Grand Salon Opera AB

Session Chair: David Malkin, The Hospital for Sick Children, Toronto, ON, Canada

Lightning Lectures highlight the top research that will be presented as posters. Selected abstracts from Poster Session B will be featured in this session. Presenters will have 1 minute to present 1 slide to highlight their most exciting finding. We invite you to discuss these in greater depth during the poster session.

A *C19MC*-LIN28A-MYCN oncogenic circuit driven by hijacked super-enhancers is a distinct therapeutic vulnerability in ETMRs—a lethal brain tumor
Iqra Mumal, The Hospital for Sick Children, Toronto, ON, Canada

***MECOM* dysregulation is associated with poor outcome in pediatric therapy-related myeloid neoplasms**
Tamara Lamprecht, St. Jude Children's Research Hospital, Memphis, TN

Genomic classification and prognosis in rhabdomyosarcoma: A report from the Children's Oncology Group, the Institute of Cancer Research, and the National Cancer Institute
Javed Khan, National Cancer Institute, Bethesda, MD

Beyond synthetic lethality: Multiple mechanisms can explain genetic interactions within childhood cancer
Josephine Daub, Princess Máxima Center for Pediatric Oncology, Utrecht, The Netherlands

Ewing sarcoma: A case study of clonal aneuploidy and DNA damage repair in pediatric cancer
Xiaofeng Su, Koch Institute, Massachusetts Institute of Technology, Cambridge, MA

A CRISPR/Cas9 domain screen identifies a small motif in the PAX3-FOXO1 transactivation domain relevant for tumor maintenance in alveolar rhabdomyosarcoma
Marco Wachtel, University Children's Hospital Zurich, Zurich, Switzerland

CATACOMB: An endogenous inducible gene that antagonizes H3K27 methylation activity of Polycomb Repressive complex 2 via a H3K27M-like mechanism
Andrea Piunti, Northwestern University, Chicago, IL

EWS-FLI1 orchestrates Ewing sarcoma plasticity through a post-translational modification cascade regulating FOXM1 stability
Heinrich Kovar, St. Anna Children's Cancer Research Institute, Vienna, Austria

Liaison between SNAI2 and *MYOD* enhances oncogenesis and suppresses differentiation in fusion-negative rhabdomyosarcoma
Myron Ignatius, Greehey Children's Cancer Research Institute, Department of Molecular Medicine, UT Health Sciences Center, San Antonio, TX

Modeling a pathogenic *SAMD9* mutation in human induced pluripotent stem cells
Jason Schwartz, St. Jude Children's Research Hospital, Memphis, TN

Mutant RAS represses CASZ1, a novel regulator of MYOD and MYOG, to inhibit embryonal rhabdomyosarcoma differentiation
Zhihui Liu, National Cancer Institute, Bethesda, MD

Overexpression of *TLX3* or *HOXA9* in association mutant *IL7R*α is sufficient to generate T-ALL in vivo
Gisele Rodrigues, Cancer and Inflammation Program, National Cancer Institute, Frederick, MD

Validation of a model of pedriatric leukemia based on pluripotent stem cells using mass cytometry
Joan Domingo Reines, Gene Regulation, Stem Cells and Development Group, GENYO-Centre for Genomics and Oncological Research-Pfizer/University of Granada/Junta de Andalucía, Granada, Spain

Characterizing vascular invasion in hepatoblastoma
Sarah Woodfield, Baylor College of Medicine, Houston, TX

Dissecting the heterogeneity of metastatic neuroblastoma cells by single-cell RNA-seq
Alice Shan, University of Toronto, Toronto, ON, Canada

Investigating the role of tumor:bone microenvironment crosstalk in Ewing sarcoma progression
Kelsey Temprine, University of Michigan Medical School, Ann Arbor, MI

Epigenomics and single-cell sequencing define a developmental hierarchy in Langerhans cell histiocytosis
Caroline Hutter, St. Anna Children's Cancer Research Institute, Vienna, Austria

Spatial and temporal conditions for Smarcb1 deletion determines mouse AT/RT (atypical teratoid/rhabdoid tumor) subtype
Zhi-Yan Han, Institut Curie, Paris, France

Three distinct subgroups of Wilms' tumors with novel molecular features and important clinical implications are defined by genome-wide DNA methylation profiles
Jack Brzezinski, Hospital for Sick Children, Toronto, ON, Canada

12:50 p.m.–3:15 p.m. **POSTER SESSION B / LUNCH**
Soprano Ballroom

3:15 p.m.–5:15 p.m. **PLENARY SESSION 6: PATHWAYS OF ONCOGENESIS II: BASIC BIOLOGY**
Grand Salon Opera AB

Session Chair: E. Alejandro Sweet-Cordero, University of California San Francisco, San Francisco, CA

Blocking purine synthesis in cancer promotes response to immunotherapy
Ayelet Erez, Weizmann Institute of Science, Rehovot, Israel

Tumor cells hijack diverse cellular processes to maintain redox balance
Poul H.B. Sorensen, BC Cancer Research Centre, Vancouver, BC, Canada

Lessons from a rare childhood cancer syndrome on replication repair deficiency and hypermutation
Uri Tabori, The Hospital for Sick Children, Toronto, ON, Canada

Lactate dehydrogenase A is a pharmacologically tractable EWS-FLI1 transcriptional target that regulates the glycolytic dependence of Ewing sarcoma*
Christine Heske, National Cancer Institute, Bethesda, MD

STAG2* mutations alter topologic organization of the genome and cis-mediated interactions
Didier Surdez, Institut Curie, Paris, France

5:15 p.m.-6:30 p.m. **SPECIAL SESSION 3: HOT TOPICS IN PEDIATRIC CANCER RESEARCH FROM HIGHLY RATED ABSTRACTS**
Grand Salon Opera AB

Session Chair: Adam Shlien, The Hospital for Sick Children, Toronto, ON, Canada

Disruption of IL6-mediated paracrine signaling to prevent pulmonary metastasis*
John Hinckley, The Ohio State University, Nationwide Children's Hospital, Columbus, OH

EP300 controls the oncogenic enhancer landscape of high-risk neuroblastoma*
Adam Durbin, Dana-Farber Cancer Institute, Boston, MA

IL6-mediated self-seeding functions to prevent osteosarcoma metastasis*
Amy Gross, Nationwide Children's Hospital, Columbus, OH

Surgical excision of the primary tumor in osteosarcoma model results in enhanced metastatic growth by modulating the lung immune microenvironment*
Michelle Kallis, The Elmezzi Graduate School of Molecular Medicine, Northwell Health, Karches Center for Oncology, The Feinstein Institute for Medical Research and Donald and Barbara Zucker School of Medicine at Hofstra/Northwell, Department of Surgery, Manhasset, NY

Ewing sarcoma cells exploit the IL1RAP-CTH axis to drive oxidative stress adaptation and lung metastasis*
Haifeng Zhang, University of British Columbia, Vancouver, BC, Canada

6:30 p.m. **EVENING ON OWN**

**Short talk from proffered abstract*

CONFERENCE PROGRAM

Friday, September 20

7:00 a.m.–8:00 a.m. **BREAKFAST AND NETWORKING ROUNDTABLES**
Grand Salon Opera C

8:00 a.m.–9:45 a.m. **PLENARY SESSION 7: SURVIVORSHIP RESEARCH**
Grand Salon Opera AB

Session Chair: Gregory T. Armstrong, St. Jude Children's Research Hospital, Memphis, TN

The lifetime impact of cancer and cancer therapy
Gregory T. Armstrong

Optimizing cancer survivorship outcomes with intervention, dissemination, and implementation research
Tara O. Henderson, University of Chicago, Chicago, IL

The need for evidence-based survivorship care
Leontien C.M. Kremer, Princess Máxima Center for Pediatric Oncology, Utrecht, The Netherlands

Acting at a distance: Medulloblastoma secreted ligands disrupt normal neural stem cell function*
Alexander Gont, The Hospital for Sick Children, Toronto, ON, Canada

9:45–10:15 a.m. **BREAK**
Grand Salon Opera Foyer

10:15 a.m.–12:45 p.m. **PLENARY SESSION 8: TUMOR HETEROGENEITY**
Grand Salon Opera AB

Session Chair: Sam Behjati, Wellcome Sanger Institute, Cambridge, United Kingdom

Comprehensive transcriptomic characterization of 1,400 sarcomas for diagnosis and immune contexture*
Julien Vibert, Institut Curie, Paris, France

Immunogenomic landscape of pediatric solid malignancies*
Jun Wei, National Cancer Institute, Bethesda, MD

Patient-derived organoids in pediatric cancer research
Jarno Drost, Princess Máxima Center for Pediatric Oncology, Utrecht, The Netherlands

Tumor uniformity
Sam Behjati

Developmental and oncogenic programs in pediatric brain tumors dissected by single-cell RNA-sequencing
Mariella G. Filbin, Dana-Farber Cancer Institute, Boston, MA

**Short talk from proffered abstract*

Using single-cell, high-dimensional approaches to unravel tumor heterogeneity in pediatric cancer
Kara L. Davis, Stanford University School of Medicine, Stanford, CA

Stalled developmental programs at the root of K27M mutant gliomas and other pediatric brain tumors
Nada Jabado, McGill University, Montreal, QC, Canada

12:45 p.m.–1:00 p.m. **CLOSING REMARKS**
Grand Salon Opera AB

Crystal L. Mackall, Stanford University School of Medicine, Stanford, CA

David Malkin, The Hospital for Sick Children, Toronto, ON, Canada

Stefan M. Pfister, Hopp Children's Cancer Center at the NCT Heidelberg (KiTZ) and German Cancer Research Center, Heidelberg, Germany

Kimberly Stegmaier, Dana-Farber Cancer Institute, Boston, MA