

## Conference Program

**Sunday, November 3**

6:30 p.m.-8:30 p.m.

**Welcome and Keynote Talks**

California Ballroom

*Session Chairperson: Lee J. Helman, National Cancer Institute, Bethesda, MD*

**Childhood cancer as discovery: More about two hits\***

Alfred G. Knudson, Fox Chase Cancer Center, Philadelphia, PA

**Gene transcription and cancer**

Stuart H. Orkin, Dana-Farber Cancer Institute, Boston, MA

**Now the hard part: Translating recent advances into improved childhood cancer outcomes**

John M. Maris, The Children's Hospital of Philadelphia, Philadelphia, PA

8:30 p.m.-10:00 p.m.

**Opening Reception**

Garden Terrace

\*An extended abstract is available in the Invited Abstracts section of the Conference Proceedings.

**PEDIATRIC CANCER AT THE CROSSROADS • November 3-6, 2013 • San Diego, CA**

## Monday, November 4

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

### **Continental Breakfast**

California Ballroom Foyer

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

### **Plenary Session 1: Developmental Biology of Pediatric Malignancies**

California Ballroom

*Session Chairpersons: Jason M. Shohet, Baylor College of Medicine, Houston, TX, and Michael A. Dyer, St. Jude Children's Research Hospital, Memphis, TN*

#### **EWS-FLI1, polycomb proteins, and stem cells: Clues to the developmental origins of Ewing sarcoma\***

Elizabeth R. Lawlor, University of Michigan, Ann Arbor, MI

#### **Targeting the oxidative stress pathway in rhabdomyosarcoma\***

Michael A. Dyer

#### **Cellular mechanisms of neuroblastoma pathogenesis: Lessons learned from the developing zebrafish**

A. Thomas Look, Dana-Farber Cancer Institute, Boston, MA

#### **H-RasG12V overexpression in the central nervous system leads to expansion of oligodendrocyte precursor cells and neural crest cells\*\***

Martin H. Distel, University of California, San Diego, CA

#### **Negative regulation of myogenesis by mTOR: A pathway toward differentiation therapy in rhabdomyosarcoma\*\***

Raphael A. Wilson, University of Texas Southwestern Medical Center, Dallas, TX

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

### **Break**

California Ballroom Foyer

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\*\*Short talks from proffered papers

10:30 a.m.-12:30 p.m.      **Plenary Session 2: Genetic Predisposition to Pediatric Cancer**

California Ballroom

*Session Chairperson: Stella M. Davies, Cincinnati Children's Hospital Medical Center, Cincinnati, OH*

**Genetic predispositions to pediatric cancers\***

Mignon L. Loh, University of California, San Francisco, CA

**Ataxia-telangiectasia: Broad implications from the study of a rare disease\***

Michael B. Kastan, Duke Cancer Institute, Durham, NC

**Fanconi anemia as a model of carcinogenesis**

Stella M. Davies

**Fanconi anemia and novel drug targets\***

Alan D. D'Andrea, Dana-Farber Cancer Institute, Boston, MA

12:30 p.m.-2:30 p.m.      **Poster Session A with Lunch**

San Diego Ballroom

2:30 p.m.-4:30 p.m.      **Plenary Session 3: Pediatric Cancer Genomics and Epigenomics**

California Ballroom

*Session Chairperson: James R. Downing, St. Jude Children's Research Hospital, Memphis, TN*

**The St. Jude Children's Research Hospital–Washington University Pediatric Cancer Genome Project: Lessons learned\***

James R. Downing

**The application of genomics to identify diagnostic biomarkers, drivers, and therapeutic targets for pediatric cancers**

Javed Khan, National Cancer Institute, Bethesda, MD

**SWI/SNF complexes are frequently mutated in cancer: Epigenetic mechanisms and therapeutic targeting\***

Charles W. M. Roberts, Dana-Farber Cancer Institute, Boston, MA

**The genomic landscape of diffuse intrinsic pontine glioma and pediatric non-brainstem high-grade glioma\*\***

Alexander K. Diaz, St. Jude Children's Research Hospital, Memphis, TN

**Novel somatic and germline mutations in intracranial germ cell tumors\*\***

Ching C. Lau, Texas Children's Hospital, Houston, TX

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## Program

4:30 p.m.-6:00 p.m.

### **Panel Discussion: Clinical Genomics**

California Ballroom

*Moderators: James R. Downing, St. Jude Children's Research Hospital, Memphis, TN, and Lee J. Helman, National Cancer Institute, Bethesda, MD*

Panelists will discuss the issue of precision/personalized medicine in pediatric cancer. The discussion will center around several key issues, questions, and challenges that emerge from the increase in genomic data available to patients and clinicians from sources ranging from private labs to large research centers. A particular focus will be the impact of this genomic data on current clinical practice. Discussion will center on how to develop a coordinated effort to leverage the vast amount of data generated from diverse sources and move forward towards a greater and more coordinated use of genomic data, particularly in developing evidenced-based protocols to drive clinical decisions in the treatment of pediatric cancer.

Panelists:

Vincent Miller, Foundation Medicine Inc., Cambridge, MA

Katherine A. Janeway, Dana-Farber Cancer Institute, Boston, MA

Donald W. Parsons, Baylor College of Medicine, Houston, TX\*

6:00 p.m.-

### **Evening Off / Dinner on Own**

## **Tuesday, November 5**

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

### **Continental Breakfast**

California Ballroom Foyer

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

### **Plenary Session 4: Brain Tumors**

California Ballroom

*Session Chairperson: Michael B. Kastan, Duke Cancer Institute, Durham, NC*

#### **Targeting placental growth factor/neuropilin 1 pathway inhibits growth and spread of medulloblastoma\***

Rakesh K. Jain, Massachusetts General Hospital, Boston, MA

#### **Mapping cancer origins\***

Richard J. Gilbertson, St. Jude Children's Research Hospital, Memphis, TN

#### **Epigenetic rewiring of pediatric glioblastomas**

Nada Jabado, McGill University, Montreal, QC, Canada

#### **Targeted therapies for pediatric brain tumors: Hope on the horizon**

Maryam Fouladi, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

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

### **Break**

California Ballroom Foyer

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

**Plenary Session 5: Leukemia**

California Ballroom

*Session Chairperson: Mignon Loh, University of California, San Francisco, CA*

**Targeting histone-modifying enzymes in leukemia\***

Scott A. Armstrong, Memorial Sloan-Kettering Cancer Center, New York, NY

**Targeting hyperactive Ras signaling in childhood cancer**

Kevin M. Shannon, University of California, San Francisco, CA

**The genomic landscape of acute lymphoblastic leukemia\***

Charles G. Mullighan, St. Jude Children's Research Hospital, Memphis, TN

**Clonal evolution enhances leukemia propagating cell activity in T-cell acute lymphoblastic leukemia through AKT/mTORC1 pathway activation\*\***

Jessica S. Blackburn, Massachusetts General Hospital, Charlestown, MA

**T cells engineered with a chimeric antigen receptor (CAR) targeting CD19 (CTL019) produce complete responses and long-term persistence without GVHD in children with relapsed, refractory ALL\*\***

David M. Barrett, The Children's Hospital of Philadelphia, Philadelphia, PA

12:30 p.m.-2:00 p.m.

**Lunch on Own**

2:00 p.m.-4:00 p.m.

**Plenary Session 6: Neuroblastoma**

California Ballroom

*Session Chairperson: John M. Maris, The Children's Hospital of Philadelphia, Philadelphia, PA*

**Targeting MYCN-driven neuroblastoma using novel therapeutic approaches\***

Michelle Haber, Children's Cancer Institute Australia for Medical Research, Sydney, Australia

**Understanding neuroblastoma flexibility**

Rogier Versteeg, Academic Medical Center, Amsterdam, The Netherlands

**ALK inhibition strategies for neuroblastoma**

Yael P. Mosse, The Children's Hospital of Philadelphia, Philadelphia, PA

**Knock-in mice with activated Alk display prolonged neurogenesis as a predisposition step to neuroblastoma\*\***

Isabelle Janoueix-Lerosey, Institut Curie, Paris, France

**Drugging MYCN protein stability through an allosteric transition in Aurora kinase A\*\***

W. Clay Gustafson, UCSF Helen Diller Family Comprehensive Cancer Center, San Francisco, CA

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\*\*Short talks from proffered papers

## Program

4:00 p.m.-5:00 p.m.      **Panel Discussion: What Advocates Can Do to Accelerate Your Research and Enhance Patient Care – A Panel on Family/Patient Advocacy in Pediatric Oncology**  
California Ballroom  
*Session Chairperson/Moderator: Susan L. Weiner, Founder, Children's Cause for Cancer Advocacy, Brooklyn, NY*

The purpose of the Family/Patient Advocacy Session is to highlight for investigators how pediatric cancer-related advocacy groups and individuals can enhance translational and clinical research. The panel will be composed of professional and parent leaders from nonprofit or research organizations actively engaged in supporting or conducting pediatric oncology research. Each member of the panel represents a different role that advocates can take in promoting research and care.

### Panelists:

LeAnn Jackson, Chair and Patient/Family Representative, Pediatric Brain Tumor Consortium Foundation, NC  
Neil Moseley, Board Member, QuadW Foundation, Dallas, TX\*  
Patrick Sullivan, President, Team Finn Foundation, Vancouver, BC, Canada  
Becky Weaver, Chief Philanthropy Officer, St. Baldrick's Foundation, Monrovia, CA  
Susan L. Weiner, Founder, Children's Cause for Cancer Advocacy, Brooklyn, NY

5:00 p.m.-7:00 p.m.      **Poster Session B with Reception**  
San Diego Ballroom

## Wednesday, November 6

7:00 a.m.-8:00 a.m.      **Continental Breakfast**  
California Ballroom Foyer

8:00 a.m.-10:00 a.m.      **Plenary Session 7: Sarcomas (Bone and Soft Tissue)**  
California Ballroom  
*Session Chairpersons: Stephen X. Skapek, UT Southwestern Medical Center, Dallas, TX, and Crystal L. Mackall, National Cancer Institute, Bethesda, MD*

**Integrating genomic approaches to identify new targets in Ewing sarcoma\***  
Kimberly Stegmaier, Dana-Farber Cancer Institute, Boston, MA

**Combination targeted therapy in pediatric sarcomas\***  
Lee J. Helman, National Cancer Institute, Bethesda, MD

**Targeting biologic drivers of metastatic disease in high-risk childhood sarcomas**  
Poul H. B. Sorensen, BC Cancer Agency Research Centre, Vancouver, BC, Canada

**High-throughput chemical screening identifies a novel class of proteasome inhibitors with significant activity against Ewing sarcoma\*\***  
Neerav N. Shukla, Memorial Sloan-Kettering Cancer Center, New York, NY

**Inhibition of deacetylase SIRT1 offers a novel treatment option in metastatic Ewing sarcoma\*\***  
Heinrich Kovar, Children's Cancer Research Institute, Vienna, Austria

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\*\*Short talks from proffered papers

10:00 a.m.-10:15 a.m.      **Break**  
California Ballroom Foyer

10:15 a.m.-12:15 p.m.      **Plenary Session 8: Opportunities and Challenges in Pediatric Oncology Translational Science and Clinical Trials**  
California Ballroom  
*Session Chairperson: Stephan A. Grupp, The Children's Hospital of Philadelphia, Philadelphia, PA*

**Emerging immune-based therapies for childhood cancer**  
Crystal L. Mackall, National Cancer Institute, Bethesda, MD

**Opportunities and challenges in pediatric oncology translational research: New drug development\***  
Susan M. Blaney, Texas Children's Hospital, Houston, TX

**NKOC-ITHER: Biology-driven individualized targeted therapy for incurable HR pediatric cancer patients**  
Hubert N. Caron, Academic Medical Center/Emma Children's Hospital, Amsterdam, The Netherlands

**Evaluating the implementation and utility of clinical tumor exome sequencing in the pediatric oncology clinic: Early results of the BASIC3 study\*\***  
Donald W. Parsons, Baylor College of Medicine Cancer Center, Houston, TX

**Overcoming immune evasion in pediatric hematologic and solid tumor malignancies: A pre-clinical study using a humanized anti-CD47 antibody\*\***  
Siddhartha S. Mitra, Stanford University, Stanford, CA

12:15 p.m.-12:30 p.m.      **Closing Remarks / Departure**  
California Ballroom

John M. Maris, The Children's Hospital of Philadelphia, Philadelphia, PA

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\*\*Short talks from proffered papers

## UPCOMING CONFERENCES

**Sixth Conference on The  
Science of Cancer Health  
Disparities in Racial/Ethnic  
Minorities and the Medically  
Underserved**

*Co-Chairpersons: John D. Carpten, Christopher I. Li, and Olufunmilayo I. Olopade*  
December 6-9, 2013  
Atlanta, GA

**CTRC-AACR San Antonio  
Breast Cancer Symposium**

*Co-Directors: Carlos L. Arteaga, C. Kent Osborne, and Peter M. Ravdin*  
December 10-14, 2013  
San Antonio, TX

**AACR-IASLC Joint  
Conference on the Molecular  
Origins of Lung Cancer**

*Co-Chairpersons: Roy Herbst, Elisabeth Brambilla, Pasi Jänne, and William Pao*  
January 6-9, 2014  
San Diego, CA

**AACR-Prostate Cancer  
Foundation Conference on  
Advances in Prostate Cancer  
Research**

*Co-Chairpersons: Arul M. Chinnaiyan, William G. Nelson, June M. Chan, and Jonathan W. Simons*  
January 18-21, 2014  
San Diego, CA

**Cancer Susceptibility and  
Cancer Susceptibility  
Syndromes**

*Co-Chairpersons: Alan D. D'Andrea, Phillip A. Dennis, and Pier Paolo Pandolfi*  
January 29-February 1, 2014  
San Diego, CA

**RAS Oncogenes: From  
Biology to Therapy**

*Co-Chairpersons: Frank McCormick, Dafna Bar-Sagi, and Channing J. Der*  
February 24-27, 2014  
Lake Buena Vista, FL

**Cellular Heterogeneity in the  
Tumor Microenvironment**

*Co-Chairpersons: Mary Helen Barcellos-Hoff, Michele De Palma, and M. Celeste Simon*  
February 26-March 1, 2014  
San Diego, CA

**AACR Annual Meeting 2014**

*Program Committee  
Chairperson: Scott W. Lowe*  
April 5-9, 2014  
San Diego, CA

**Pancreatic Cancer**

*Co-Chairpersons: Dafna Bar-Sagi, David A. Tuveson, Christine Iacobuzio-Donahue, and Alec Kimmelman*  
May 18-21, 2014  
New Orleans, LA

**AACR Precision Medicine  
Series: Drug Sensitivity and  
Resistance: Improving Cancer  
Therapy**

*Co-Chairpersons: Gideon Bollag, Elaine Mardis, Gordon Mills, and David Solit*  
June 18-21, 2014  
Orlando, FL

**Targeting the PI3K-mTOR  
Network in Cancer**

*Co-Chairpersons: Lewis C. Cantley, Jose Baselga, Joan S. Brugge, Brendan J. Manning, and Malte Peters*  
September 14-17, 2014  
Philadelphia, PA

**Hematological Malignancies**

*Co-Chairpersons: Kenneth C. Anderson, Scott Armstrong, Riccardo Dalla-Favera, and Margaret Shipp*  
September 20-23, 2014  
Philadelphia, PA

**Melanoma**

*Co-Chairpersons: Levi A. Garraway, Keith T. Flaherty, and Suzanne L. Topalian*  
September 20-23, 2014  
Philadelphia, PA

**13th Annual International  
Conference on Frontiers in  
Cancer Prevention Research**

*Program Committee  
Chairperson: Phillip A. Dennis*  
September 28-October 1, 2014  
New Orleans, LA

**EORTC-NCI-AACR  
International Symposium on  
Molecular Targets and Cancer  
Therapeutics**

*Scientific Committee Co-Chairpersons: Jean-Charles Soria, Lee J. Helman, and Jeffrey A. Engelman*  
November 18-21, 2014  
Barcelona, Spain

**Tumor Immunology**

*Co-Chairpersons: Robert H. Vonderheide, Nina Bhardwaj, Stanley Riddell, and Cynthia L. Sears*  
December 1-4, 2014  
Orlando, FL

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program updates.