



NAPAAC 2018 Symposium & Working Meeting
The Children's Hospital of Pittsburgh/UPMC
Rangos Research Center (44th St. entrance)
4401 Penn Avenue, Pittsburgh, PA15224
Wednesday, May 2nd, 2018

NAPAAC 2018 Bone Marrow Failure Research Symposium & NAPAAC 2018 Working Meeting

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Symposium [Rangos Auditorium, 3rd Floor]:

8:45 - 9:15 Registration in conference center lobby (Sign in, Name tag, Storage of luggage)

9:15 - 9:30 Welcome and introduction (Linda McAllister and NAPAAC EC)

9:30 - 10:15 Oral abstract session #1 (moderated by Taizo Nakano)

Richa Sharma (Indiana University)

"Error-prone mitosis due to impairment of the novel FANCA-BRCA2-BUBR1 spindle checkpoint signaling axis drives chromosomal instability and malignant hematopoiesis in Fanconi anemia"

Bonnie Lau (Johns Hopkins University)

"Aberrant Hedgehog Signaling in Myelodysplastic Syndrome Accelerates Leukemic Transformation via Smoothed-dependent and Smoothed-independent Gli1 Activation"

Inga Hofmann (University of Wisconsin)

"Congenital thrombocytopenia and myelofibrosis due to germline mutations in G6b-B"

10:15 - 10:25 Coffee break/Introduction to UPMC BMF program (Steven Allen)

10:25 - 11:10 Oral abstract session #2 (moderated by Grzegorz Nalepa)

Ji Zha (Children's Hospital of Philadelphia)

"Disease-specific Alterations in Bone Marrow Niche Function Influence Donor Engraftment Efficiency in Mouse Models of Inherited Bone Marrow Failure"

Emily Southard (Children's Hospital Colorado)

"The Emerging Role of MECOM Mutations in Pediatric Bone Marrow Failure: A Case Series"

Yigal Dror (The Hospital for Sick Children)

"Re-analysis of next generation sequencing gene panel data by normalized coverage values reveals previously undetected copy number variations in inherited bone marrow failure syndromes"

11:10 - 12:00 Keynote Speaker: Vijay Sankaran (Boston Children's Hospital)

"Insights into Aplastic Anemia Treatment from Human Genetic Studies"



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12:00 - 1:00 Poster session & Lunch

David Claassen (St. Jude Children's Research Hospital)

"Characterization, Treatment and Toxicities in a Pediatric Diamond Blackfan Anemia Cohort"

Inga Hofmann (University of Wisconsin)

"Congenital thrombocytopenia and myelofibrosis due to germline mutations in G6b-B"

Susan Kirk (Baylor College of Medicine)

"Transient pancytopenia subsequently diagnosed with acute leukemia: A report of three cases"

Peter Kurre (Oregon Health & Science University)

"Deficits in fetal liver expansion are the origin of a constrained HSC pool in Fancc2 mice"

Bonnie Lau (Johns Hopkins University)

"Aberrant Hedgehog Signaling in Myelodysplastic Syndrome Accelerates Leukemic Transformation via Smoothed-dependent and Smoothed-independent Gli1 Activation"

Alex Lion (Indiana University)

"A nearly missed case of Shwachman Diamond Syndrome: Social instability, a physician parent, and whole exome sequencing"

Joseph Oved (Children's Hospital of Philadelphia)

"Outcomes of Unrelated Donor Peripheral Stem Cell Transplantation for Patients with Non-malignant Hematologic Disorders Using Two Partial T cell Depletion Strategies"

Kristin Shimano (UCSF Benioff Children's Hospital)

"Transplant During Infancy for a Patient with Diamond-Blackfan Anemia with Severe Neutropenia"

Elizabeth Sierra Potchanant (Indiana University)

"A Patient-tailored Mouse Model to Enable Development of Precision Therapies for Barth Syndrome"

Emily Southard (Children's Hospital Colorado)

"The Emerging Role of MECOM Mutations in Pediatric Bone Marrow Failure: A Case Series"

Marcin W. Wlodarski (St. Jude Children's Research Hospital)

"Congenital Pure Red Cell Aplasia Caused by Deficiency of Adenosine Deaminase 2"

Ji Zha (Children's Hospital of Philadelphia)

"Disease-specific Alterations in Bone Marrow Niche Function Influence Donor Engraftment Efficiency in Mouse Models of Inherited Bone Marrow Failure"