

New Directions in Bone Marrow Failure Symposium
Texas Children's Cancer and Hematology Centers, Houston, TX

Agenda

Wednesday Sept 25

7:00-9:00 pm Buffet dinner, Hilton Houston Medical Center, Travis A dining room

Thursday Sept 26

7:30-8:00 Escort to Texas Children's Hospital Feigin Center and continental breakfast
(7:20 am meet in the lobby of the Hilton to walk over to TCH as a group)

8:00-8:15 Alison Bertuch, MD, PhD, Baylor College of Medicine and Texas Children's Hospital

Introductory remarks

Session 1

Colin Sieff, MD, Moderator, Harvard Medical School, Dana Farber Cancer Institute and Boston Children's Hospital

8:15-8:40 Adrianna Vlachos, MD, Hofstra University School of Medicine and Steven and Alexandra Cohen Children's Medical Center of New York

New advances in the genetics of Diamond Blackfan anemia (and more...)

8:40-8:50 Discussion

8:50-9:15 Jeffrey Lipton, MD, PhD, Hofstra University School of Medicine and Steven and Alexandra Cohen Children's Medical Center of New York

Exploiting mouse models to understand the pathophysiology of Diamond Blackfan anemia

9:15-9:25 Discussion

9:25-9:50 Peter Kurre, MD, Oregon Health and Science University School of Medicine and Doernbecher Children's Hospital

Developmental programming of hematopoietic failure in Fanconi anemia

9:50-10:00 Discussion

10:00-10:20 Coffee break

Session 2

Phoenix Ho, MD, Moderator, University of Washington School of Medicine, Fred Hutchinson Cancer Research Center, and Seattle Children's Hospital

10:20-10:45 Akiko Shimamura, MD, PhD, University of Washington School of Medicine, Fred Hutchinson Cancer Research Center and Seattle Children's Hospital

Marrow failure: Lessons from Shwachman-Diamond syndrome

10:45-10:55 Discussion

10:55-11:20 Yigal Dror, MD, FRCPC, University of Toronto, The Hospital for Sick Children, Toronto, and Institute of Medical Sciences

Advances in understanding the hematopoietic defects in Shwachman-Diamond syndrome

- 11:20-11:30 Discussion
- 11:30-12:05 Gary Kupfer, MD, Yale University School of Medicine, Smilow Cancer Hospital at Yale and Yale-New Haven Children's Hospital
Fanconi anemia: interactions with transcription and trans lesion synthesis repair
- 12:05-12:15 Discussion
- 12:15-1:45 Lunch for invited speakers, TCH, CNRC lobby
- Session 3** Helge Hartung, MD, Moderator, University of Pennsylvania Perelman School of Medicine, and The Children's Hospital of Philadelphia
- 1:45-2:10 Alison Bertuch, MD, PhD, Baylor College of Medicine and Texas Children's Hospital
Patient as muse: new insights into molecular mechanisms of bone marrow failure
- 2:10-2:20 Discussion
- 2:20-2:45 Seth Corey, MD, MPH, Northwestern University Feinberg School of Medicine, Ann & Robert Lurie Children's Hospital of Chicago and Robert H. Lurie Comprehensive Cancer
Myelodysplasia associated with severe congenital neutropenia: a game of clones
- 2:45-2:55 Discussion
- 2:55-3:20 Inga Hofmann, MD, Harvard Medical School, Dana Farber Cancer Institute and Boston Children's Hospital
GATA2 mutations in pediatric myelodysplastic syndrome and bone marrow failure
- 3:20-3:30 Discussion
- 3:30-3:50 Coffee break
- Session 4** Ulrike Reiss, MD, Moderator, St Jude Children's Research Hospital
- 3:50-4:15 Timothy Olson, MD, PhD, University of Pennsylvania Perelman School of Medicine, and Children's Hospital of Philadelphia
Targeting the BM HSC niche to improve engraftment following BMT for bone marrow failure
- 4:15-4:25 Discussion
- 4:25-4:50 Clifford Takemoto, MD, and Christopher Gamper, MD, PhD, Johns Hopkins School of Medicine and Johns Hopkins Children's Center, Division of Pediatric Hematology

High dose cyclophosphamide for pediatric severe aplastic anemia-the Johns Hopkins experience

- 4:50-5:00 Discussion
- 5:00-5:25 Leslie Lehmann, MD, Harvard Medical School, Dana Farber Cancer Institute and Boston Children's Hospital
The role of BMT in pediatric severe aplastic anemia: in the beginning, in the middle, in the end or never
- 5:25-5:35 Discussion
- 5:35-6:05 Leslie Lehmann, MD;
James Huang, MD, University of California, San Francisco School of Medicine and UCSF Benioff Children's Hospital;
Timothy Olson, MD, PhD
BMT panel discussion
- 6:15-8:30 Dinner for invited speakers, TCH, CNRC lobby
(8:30 pm meet at the front door of the CNRC to be transported back to the hotel)

Friday Sept 27

- 7:45-8:30 Escort to Texas Children's Hospital Feigin Center and continental breakfast
(7:30 am meet in the lobby of hotel with your luggage to be transported over to the Feigin Center)

Session 5

- Kelsie Storm, MD, Moderator, Oregon Health and Science University School of Medicine, Doernbecher Children's Hospital
- 8:30-8:55 Bertil Glader, MD, PhD, Stanford University Medical Center and Lucile Packard Children's Hospital at Stanford
eADA, then and now
- 8:55-9:05 Discussion
- 9:05-9:30 Anupama Narla, MD, Stanford University Medical Center and Lucile Packard Children's Hospital at Stanford
Novel therapeutic strategies for Diamond Blackfan anemia and other ribosomopathies
- 9:30-9:40 Discussion
- 9:40-10:00 Coffee break

Session 6

- James Huang, MD, Moderator
- 10:00-10:25 Katherine King, MD, PhD, Baylor College of Medicine and Texas Children's Hospital
Interferon: cellular pathophysiology of bone marrow failure
- 10:25-10:35 Discussion

- 10:35-11:30 Tarek Elghetany, MD, Baylor College of Medicine and Texas Children's Hospital;
Inga Hofmann, MD, Harvard Medical School, Dana Farber Cancer Institute and
Boston Children's Hospital;
Michele Paessler, MD, University of Pennsylvania Perelman School of Medicine,
Children's Hospital of Philadelphia
Hematopathology session
- 11:30-12:00 Anjulika Chawla, MD, The Warren Alpert Medical School of Brown University and
Hasbro Children's Hospital;
Ulrike Reiss, MD, St. Jude Children's Research Hospital;
Zora Rogers, MD, UT Southwestern Children's Medical Center at Dallas;
Kelly Walkovich, MD, University of Michigan Medical School and C.S. Mott
Children's Hospital
Supportive care panel discussion
- 12:15-3:00+ Lunch for invited speakers & NAPAAC discussions, on-scope hematopathology
discussions & TCH tours, departures