

HCT
in
Neurometabolic Diseases

August 11 and 12, 2009
Beaver Hollow Conference Center
Java Center, New York

Sponsored by:

HRSA (Maternal and Child Health Bureau)

and

The Hunter's Hope Foundation

Session 1

Tuesday, August 11, 2009

HCT in Neurometabolic Diseases
How Do Results Compare With those of Krabbe Disease?

Co-Chairs: Richard Erbe, MD (Geneticist, University of Buffalo)
Kirk Schultz, MD (Transplanter, BC Children's Hospital, Vancouver)

- 7:00-8:00 Breakfast
- 8:00-8:30 Introduction and Review of the 2008 Workshop (Patti Duffner, MD)
- 8:30-8:50 The Krabbe Disease Transplant Experience (Joanne Kurtzberg, MD)
- 8:50-9:05 Discussion
- 9:05-9:25 The MPS 1 transplant experience (Jakub Tolar, MD, PhD)
- 9:25-9:40 Discussion
- 9:40-10:00 The MLD Transplant Experience (Larry Charnas, MD, PhD)
- 10:00-10:15 Discussion
- 10:15-10:35 The ALD Transplant Experience (Paul Orchard, MD)
- 10:35-10:50 Discussion
- 10:50-11:10 The Nieman Pick Syndrome Transplant Experience (Marc Patterson, MD)
- 11:10-11:25 Discussion
- 11:25-11:45 Lysosomal Storage Disorders where HCT is Ineffective (Chet Whitley, MD, PhD)
- 11:45-12:30 Discussion
- 12:30-1:30 Lunch

Session 2

Tuesday, August 11, 2009

HCT in Neurometabolic Diseases
Late Effects

- Co-Chairs: Verne Caviness, MD, D.Phil. (Neurologist, MGH)
Mark Noble, PhD (Biomedical Geneticist, University of Rochester)
- 1:30-1:50 Long Term Outcomes of HCT in Infants with Leukemia (Jean Sanders, MD)
- 1:50-2:05 Discussion
- 2:05-2:25 Long Term Endocrine Effects following HCT in Infants (Chuck Sklar, MD)
- 2:25-2:40 Discussion
- 2:40-3:00 Long Term Neurocognitive and Neurobehavioral Effects of HCT in Lysosomal Diseases (Elsa Shapiro, PhD., ABPP, LP)
- 3:00-3:15 Discussion
- 3:15-3:35 Long Term Effects of HCT on Pulmonary Function and the Development of Second Malignancies (David Mitchell, MD)
- 3:35-3:50 Discussion
- 3:50-4:10 Long Term Effects of Chemotherapy in Animal Models (Mark Noble, PhD)
- 4:10-4:20 Long Term Effects of Bone Marrow Transplantation (Chemotherapy) on Twitcher and Wild Type Mice (Yoichi Kondo, MD)
- 4:20-4:35 Discussion
- 4:35-4:55 Neuroprotection (Ernesto Bongarzone, PhD)
- 4:55-5:10 Discussion

Session 3

Wednesday, August 12, 2009

The Need for a Standardized Protocol for the Evaluation and Long-term Follow-up of Children Transplanted for EIKD

- Co-Chairs Marc Patterson, MD (Neurologist, Mayo Clinic)
Chester Whitley, MD, PhD (Geneticist, University of Minnesota)
- 8:00-8:15 Nosology of GLD/KD (Larry Charnas, MD, PhD)
- 8:15-8:30 Inclusion /exclusion Criteria for HCT in Presymptomatic & Symptomatic Infants (Joanne Kurtzberg, MD)
- 8:30-8:45 Referral for HCT in Children with Positive NBS/ Confirmatory Testing the NYS Model (Patti Duffner, MD)
- 8:45-9:00 Discussion
- 9:00-9:15 Neurodiagnostic Testing (NCV, BAER, VER, LP, Biomarkers)
- 9:15-9:30: MRI [Loes scale] (Jim Provenzale, MD)
- 9:30-9:45 Discussion
- 9:45-10:00 Developmental /Functional evaluations (Mike Msall, MD)
- 10:00-10:15 Discussion
- 10:15-10:35 Neuropsychological Assessments the COG Model (Paul Fisher, MD)
- 10:35-10:50 Discussion
- 10:50-11:05 Endocrine Evaluation (Chuck Sklar, MD)
- 11:05-11:20 Discussion
- 11:20-11:35 Pulmonary Toxicity, Late Malignancies (David Mitchell, MD)
- 11:35-11:50 Discussion
- 11:50-12:30 Final Thoughts. Where do we go from here?