ECD Studies and Trials Panel
2016 Patient & Family Gathering
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Treating and Research of Histiocytic Diseases
Children 40 years  Adults 27 years
Houston, Texas
USA
Current Research Projects/Clinical Trials

• Whole exome sequencing of biopsy specimens
• Proteomic signature of plasma/Spinal Fluid
• Assay of Spinal Fluid for mutated cells
• Clinical Trial of second generation BRAF inhibitor
• Clinical Trial of combination inhibitor
Why be involved & Why are these studies important?

• Advance knowledge of ECD biology
• Determine efficacy of new treatments
What do patients do, who is eligible?

• Come to Houston for a consult or enroll remotely
  send blood or CSF specimen
• Eligibility: Basic research studies- all ECD patients
  Clinical: active ECD and no therapy for a month
What is Expected of Patients

• Travel to Houston: Once for Research studies  
  Clinical trials: to be determined  

• Patients are responsible for travel, office visit. Research tests are not charged. Drugs may be provided by company

• Patients outside the USA may participate in research studies

• Clinical trial: need to have a companion  

• Length of stay dependent on clinical condition and ease of collaboration with local provider
Planned Clinical Trials

• New BRAF inhibitor
• Combination inhibitor

Discussions with Pharmaceutical companies ongoing.