

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



Baylor
College of
Medicine

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.

