

September 16, 2016

4<sup>th</sup> Annual ECD Patient and Family Gathering

# **ECD Studies at MSKCC**

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Memorial Sloan Kettering  
Cancer Center

# Phase 2 Trial of Cobimetinib for Adults with Histiocytic Disorders

- **Clinical Trial for ECD patients WITHOUT *BRAF* mutation**
- **Or for BRAF+ who cannot obtain or cannot take BRAF inhibitors**
- **7 out of 7 patients treated thus far with improvement**
- **11 spots remaining**
- **Medication is provided by company**

# **Pilot Study of 18F-Fluorocholine PET to Improve Accuracy of Diagnostic Biopsy in Histiocytic Disorders**

- **Testing new kind of PET scan performed when biopsy is done to diagnose ECD or to obtain tissue for genetic testing**
- **PET scan uses choline which is used by cells that are dividing**
- **Hopefully this PET will identify ECD cells more specifically than “regular” FDG PET alone**
- **Participants get both PET scans and biopsy of two ECD sites and results are compared**
- **Three patients enrolled, two spots remaining**

# A Prospective Study of the Natural History and Genetic Landscape of Histiocytic Disorders

- **Biospecimen protocol**
- **ECD patients give blood, urine, CSF to be stored for research**
- **Analyzed for gene mutations, markers of inflammation**
- **54 ECD patients enrolled, >200 samples**

# **A Clinical, Structural, and Functional Neuroimaging Study of Cognition in Adults with Histiocytosis**

- **Detailed study of brain function in patients with ECD (funded by ECDGA)**
- **Participants undergo MRI of the brain, cognitive testing, quality of life and symptom questions**
- **Trying to detect early brain changes in ECD and patterns of thinking and memory problems**
- **30 spots available**

# ECD Global Registry

- **MSKCC and Newcastle (UK)**
- **ECDGA-funded**
- **Collects clinical information, scans about ECD patients over time**
- **All ECD patients encouraged to participate**
- **Can enroll by telephone**
- **Answer surveys online about about symptoms, quality of life, ways ECD affects your lives**
- **Optional collection of blood and urine**
- **Open January 2017**

**Thank You!**