



ECD Global Alliance Patient & Family Gathering Treatment Options

Ronald S. Go, MD
Division of Hematology
Mayo Clinic, Rochester, Minnesota

October 27, 2017

New York, NY

Treatment Options

Clinical Trial

Off-Study

BRAF^{V600E} (+)

BRAF^{V600E} (-)/Unknown

BRAF Inhibitor

MEK Inhibitor

Immunotherapy

Chemotherapy

Anti-Cytokine

Other

Vemurafenib

Dabrafenib

Trametinib

Cobimetinib

Interferon

Peginterferon

Sirolimus

Cladribine

Vinblastine

Methotrexate

Anakinra

Canakinumab

Infliximab

Prednisone

Imatinib

Radiation

BRAF/MEK Inhibitors

Vemurafenib

- Oral
- Fatigue
- Rash
- Diarrhea
- Joint pain

Dabrafenib

- Oral
- Fatigue
- Rash
- Diarrhea
- Joint pain

Trametinib

- Oral
- Fatigue
- Rash
- Diarrhea
- Heart failure

Cobimetinib

- Oral
- Fatigue
- Rash
- Diarrhea
- Heart failure

Chemotherapy

Cladribine

- IV
- Fatigue
- Infection

Vinblastine

- IV
- Fatigue
- Neuropathy
- Infection

Methotrexate

- Oral or IV
- Fatigue
- Infection

Immunotherapy

Interferon

- Skin injection
- Fatigue
- Flu-like
- Nausea
- Infection

Peginterferon

- Skin injection
- Fatigue
- Flu-like
- Nausea
- Infection

Sirolimus

- Oral
- Hypertension
- High cholesterol
- Anemia
- Rash
- Diarrhea
- Infection

Anti-Cytokine (IL-1/TNF Inhibitors)

Anakinra

- Skin injection
- Headache
- Infection
- Nasopharyngitis

Canakinumab

- Skin injection
- Headache
- Infection
- Diarrhea
- Nasopharyngitis

Infliximab

- IV
- Infusion reaction
- Headache
- Infection

Clinical Trials

Country	Phase	Treatment	Institutions	Comments
USA	I	Anakinra or denosumab + everolimus (mTOR inhibitor)	MD Anderson (Houston)	NCT01624766; previously treated
USA	I	DCC-2618 (c-Kit inhibitor)	Multiple	NCT02571036 previously treated
USA	I/II	PLX8394 (BRAF inhibitor)	Multiple (11 sites)	NCT02428712; previously treated BRAF mutated
USA	II	Lenalidomide (immunomodulatory agent)	Dana Farber (Boston)	NCT02523040; newly diagnosed or previously treated
USA	II	Dabrafenib (BRAF inhibitor) or trametinib (MEK inhibitor)	National Institute of Health (Bethesda)	NCT02281760; BRAF mutated; Newly diagnosed or previously treated; study suspended
USA	II	Nivolumab (PD1 antibody)	Multiple (52 sites)	NCT02832167; previously treated

Non-Interventional Studies

Country	Type	Study	Institutions	Comments
USA	Observational, imaging, labs	Clinical and basic investigations into ECD	National Institute of Health (Bethesda)	Evaluation and natural history
Any	Retrospective	Multicenter study on patients treated with anakinra	Centre Hospitalier Universitaire Côte de Nacre (Caen; lead institution)	Collect data on safety and efficacy
France USA	Observational	Long-term outcome after vemurafenib/BRAF inhibitors interruption in ECD (LOVE)	Groupe Hospitalier Pitie-Salpetriere (Paris); Memorial Sloan Kettering (New York)	Treatment interruption in patients in remission or with stable disease
USA	Imaging	Uptake and biodistribution of 18F-fluorocholine in histiocytic disorders by PET imaging and biopsy measurement	Memorial Sloan Kettering (New York)	Imaging to distinguish reactive versus malignant histiocytic infiltration
USA	Neurocognitive testing, quality of life, imaging	A study of memory, thinking, and brain imaging in adults with histiocytosis	Memorial Sloan Kettering (New Jersey, New York)	Questionnaires and MRI in patients without brain involvement
Any	Observational	International Rare Histiocytic Disorders Registry	Hospital for Sick Children (Toronto; lead institution)	Disease registry for all patients

Thank you

go.ronald@mayo.edu