ECD Global Alliance
Patient & Family Gathering
Treatment Options

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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

<table>
<thead>
<tr>
<th>Interferon</th>
<th>Peginterferon</th>
<th>Sirolimus</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Skin injection</td>
<td>• Skin injection</td>
<td>• Oral</td>
</tr>
<tr>
<td>• Fatigue</td>
<td>• Fatigue</td>
<td>• Hypertension</td>
</tr>
<tr>
<td>• Flu-like</td>
<td>• Flu-like</td>
<td>• High cholesterol</td>
</tr>
<tr>
<td>• Nausea</td>
<td>• Nausea</td>
<td>• Anemia</td>
</tr>
<tr>
<td>• Infection</td>
<td>• Infection</td>
<td>• Rash</td>
</tr>
</tbody>
</table>

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

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

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Thank you

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