



ECD Global Alliance Patient & Family Gathering Treatment Options

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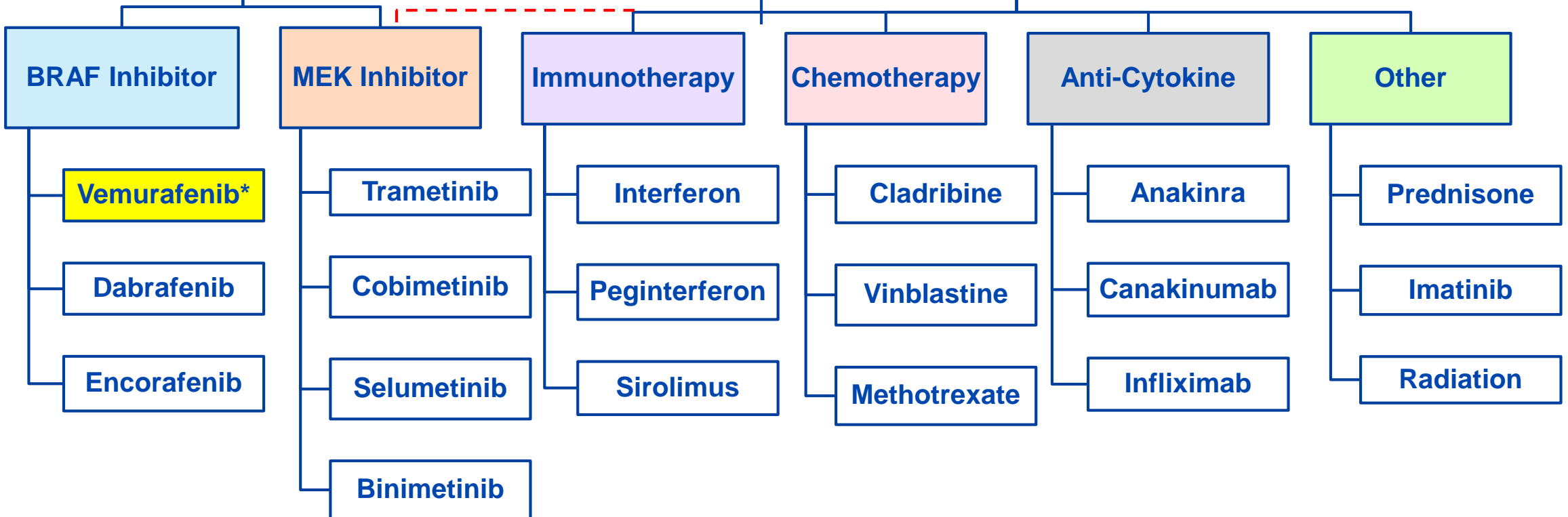
Treatment Options

Clinical Trial

Off-Study

BRAF^{V600E} (+)

BRAF^{V600E} (-)/Unknown



*FDA approved indication

BRAF/MEK Inhibitors

Vemurafenib*

- Oral
- Fatigue
- Rash
- Diarrhea
- Joint pain

Dabrafenib

- Oral
- Fatigue
- Rash
- Diarrhea
- Joint pain

Trametinib

- Oral
- Fatigue
- Rash
- Diarrhea
- Eye toxicity
- Heart failure

Cobimetinib

- Oral
- Fatigue
- Rash
- Diarrhea
- Eye toxicity
- Heart failure

*FDA approved indication

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

Chemotherapy

Cladribine

- IV
- Fatigue
- Infection

Vinblastine

- IV
- Fatigue
- Neuropathy
- Infection

Methotrexate

- Oral or IV
- Fatigue
- 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

Supportive Care

- Hormonal evaluation
- Neurocognitive assessment
- Dermatologic evaluation

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	Altiratinib (DCC-2701; multi-kinase inhibitor)	Multiple	NCT02228811; 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	Cobimetinib (MEK inhibitor)	Memorial Sloan Kettering (New York)	NCT02649972; newly diagnosed or previously treated
USA	II	Nivolumab (PD1 antibody)	Multiple (52 sites)	NCT02832167; previously treated

Thank you

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