

Current Known ECD Treatment Options

Therapy Category	Use	Drug name	Typically administered as	More Common side Effects Requiring Monitoring and/or Personal Adjustments
Targeted treatment	BRAF V600E mutation	• vemurafenib (Zelboraf) - FDA approved • dabrafenib (Tafinlar) - clinical trial with results • encorafenib (Braftovi) - off label use • HLX208 - in clinical trial - side effects yet unknown	tablet or capsule once or twice daily	Skin issues (sensitivity to sun, rash or skin cancer), fatigue, joint pain, heart issues (rhythm & pumping inefficiencies)
	MAPK mutation	• cobimetinib (Cotellic) - FDA approved • trametinib (Mekinist) - clinical trial with results • binimetinib (Mektovi) - off label use • ulixertinib (BVD-523) - under study	tablet or capsule once or twice daily	Skin issues (sensitivity to sun and rash), fatigue, joint pain, heart issues (rhythm & pumping inefficiencies), vision issues
	Other mutations	• crizotinib (Xalori) - off label use for RET-fusion • pexidartinib (Turalio or PLX- 3397) - off label use for CSF1R mutation • Q702 - early study for CSF1R mutation • larotrectinib (Vitrakvi) - off label use for NTRK fusion • entrectinib (Roxlytrek) - off label use for NTRK fusion • sirolimus (Rapamune) - off label use for PIK3CA mutation • everolimus (Afinitor & Zortexx) - off label use • selipercatinib (Retevmo) - off label use for RET fusion • imatinib (Gleevec or Gilvec) - off label use as physician decision • sorafenib (Naxavar) - off label use as physician decision	capsule or tablet once or twice daily	Varies
Chemotherapy	Physician / patient decision	• cladribine (Leustatin) - off label use • methotrexate (Rheumatrex, Trexall, Otrexup, Rasuvo) - off label use • cytarabine (ara-C, Cytosar-U, Tarabine PFS) - off label use • vinblastine (Velban, Alkaban-AQ) - off label use	• Intravenous • tablet or injection • Intravenous • Intravenous	Varies + Increased risk of infection
Immunotherapy	Physician / patient decision	• pegylated interferon alpha (Pegasys) - off label use • interferon alpha-2a (Roferon-A, Intron-A) - off label use	• Injection weekly • injection multiple times per week	Fatigue, flu-like symptoms, joint pain, depression
Biological Therapy	Physician / patient decision	• anakinra (Kineret) - off label use • canakinumab (Ilaris) - off label use • tocilizumab (Actemra) - off label use • infliximab (Remicade) - off label use	Injections at various frequencies	Varies

Bolded treatments are most commonly used today.

October 2023