

BRAF Negative Discussion

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The following is a summary of this session provided by Eli L. Diamond, MD.

Dr. Diamond gave an overview of treatment options for BRAF “negative” or “wildtype” ECD. The most commonly used treatment in this context is interferon alpha. There is a good amount of data for this treatment, certainly as compared to others, and it can be effective in reducing ECD lesions. It is an injection into the skin, either weekly or three times per week, and can have several side effects including feeling unwell and changes in mood. Anakinra is a treatment with less concrete evidence but has been used more and more in recent years. It is a daily injection into the skin and has few side effects aside from skin reactions and possibly lowering levels of some blood cells. It seems to be most effective for treating bone pain and symptoms like fatigue, fevers, and night sweats. The effectiveness of anakinra for more severe forms of ECD is not really known but it is not felt to be the best choice for these. Methotrexate is a more mild medication that can be given as a weekly pill or weekly injection. There is good evidence for its use in many ECD-like illnesses but not ECD specifically. The literature is quite limited but there are many anecdotes of patients with ECD doing well with this medication.

Dr. Diamond also explained that there has been exciting research identifying the gene mutations that are causing ECD in the BRAF-negative cases and that these mutations are identifiable in most patients if testing is done in the proper way. Furthermore, the identification of these mutations is leading to new treatment ideas. There are a small number of cases of ECD patients being treated for these non-BRAF mutations, with medications that target those mutations similarly to how Vemurafenib targets BRAF, and with similarly exciting results. There is a clinical trial of one of these medications opening at MSKCC in early December 2015.