

## **Summary of ECD Global Alliance Internet Chat** **07 Mar 2015**

8 Attendees

- A member who has dropped her vemurafenib (V) dose to 1 dose in the morning and 1 in the evening, after 15 months on higher doses, was asked how she was doing. She told us that she was doing OK with no major changes yet. She had been started on 4 a day, but only lasted on this dose for 3 weeks. She couldn't bear the pain. She has no checkup for 6 months. Her doctor said that if she feels much worse again, to up the dose. She was asked what is/are the indicators of feeling "worse" and her doctors replied that this was a good question.

She was asked to keep us posted, as probably all V users will need to go on a maintenance dose eventually, either V itself or something else. This is the reason that the urine test is going to be so useful because it can detect the onset of BRAF mutation flare-up very cost effectively.

- Another member is not allowed to lower the dose of V below 4 a day while he is on the trial. He is currently on 6 a day. But he has heard of participants taking 4 a day, every other week, with the weeks on break to recover from side effects.
- A new member came on who was diagnosed last month. She has lesions in her cerebellum, lytic and sclerotic bones and skin lesions. She has been married to a doctor for the last 32 years (but he is a psychiatrist, and ECD doesn't appear much in the psychiatric department), and she is an RN. She lives in Kentucky (3 miles from where the Breeders Cup will be run this October). She is not on any medicine for ECD, and has never had a PET scan, just an MRI to look at lesions in her cerebellum.

Two of her sisters have read a lot about ECD on line. She has come to the conclusion that there are some doctors who act like they have heard of it, but really know nothing, and that many of the local doctors' act like ECD doesn't really exist, if they haven't heard of it.

She is BRAF+, and is going to the National Institute of Health (NIH) in April. She had been to Mayo last summer, but they didn't "figure it out". Also to Duke, who figured it out by accident, and many other specialists in Lexington over the last few years. She was "ready" for someone who was knowledgeable and interested. She happened to "hit" NIH at the right time, and is going to be in the study involving 2 drugs. Recently, another member came on the chat who is also on this trial. He is doing very well.

- A member told us that Dr. Diamond was going to take her off the drug that she is on, because he didn't think it was working and that there were too many side effects. She is ARAF positive so she is the only one of us taking this drug. She had had an OCT (this is an

image that the retina specialist takes, that shows the macula and if there is any edema). Her edema had actually gotten worse. But right before they were going to give her a steroid injection, she asked for another OCT, and this time there had been an improvement in the edema, so the doctor decided to give the drug another chance, and increased the dose.

She is worried about potential side-effects. These include; high blood pressure, hand-foot syndrome, heart issues, rash, bleeding, fatigue, etc. Right now she has high blood pressure and a rash. Hopefully, in a couple of weeks, she will have another OCT and it will show even more improvement.

It was said that V has a similar side-effect profile, but in the trial using Dabrafenib and Trametinib at NIH, there are relatively less side effects than V.

- A member that had treatment with both interferon and cladribine (before they "invented" BRAF) has been on 40MG methotrexate once a week, for some years. He has been stable for a few years now. He has been having a PET-CT scan once a year. In fact, he is due to go back to the doctor this week for the result of a recent one. He will find out if he has any active disease or if he is in "remission". [Summarizer's comment; since it was me, I can tell you that the scan WAS negative - but the methotrexate continues.]  
He has not been in "remission" before and is still going to be slurry and wobbly(!), but not getting worse. He will not be able to go back to work though [hurray!].
- The new member said that this sounded like her. A good friend was talking on the phone with her last fall, and the friend called back to ask why she was slurring her speech! So, she saw a neurologist, who reluctantly did an MRI, which began her ECD journey. Up until then she was walking with her 60 lb labradoodle, about 15 miles/week. "Not anymore!" [Summarizer again; I had an 80 lb standard poodle which I had to re-home because he would pull me over!]
- A member who has been in remission with no active disease for a couple of years is seeing the doctor, because her limited sight is getting even blurrier. She had an MRI this week and goes to see the specialist on the 23rd. She is also wobbly and slurry. She has been tested for BRAF a couple of times and has been negative.
- A member mentioned the "ECD history" trial at NIH. Participants go to NIH annually or biannually, for a full evaluation, but not for treatment.
- We were asked whether we had any fevers. Another also has had low grade fevers with chills, and also had intermittent eye issues, where it looks like water waves in front of her eyes. It goes away after about 15 minutes. Recently, our new member got a sudden redness in the sclera, but it cleared up after a few hours with no treatment, and another member said that this sounded exactly like what is bothering him sometimes! Others told us about having swellings behind the eyes, and one had tumors on his optic nerves.

- A Canadian member has swelling behind one eye, but it hasn't affected his sight yet. He sees a hematologist in Canada. This doctor is looking at other treatment options, maybe V, if he can get it there. He has been in contact with the NIH, as well. He is in status quo-no better, no worse. He finished a course of chemotherapy with Cladiribine in December. One week a month of treatment for 6 months. The Cladiribine did not work as they had hoped that it would. The lesions didn't grow, but they didn't shrink either. He is BRAF positive, and it was said that he would most likely benefit from the NIH trial, although no-one was sure how they handle non-US participants. [Editor's note" non-US citizens are being accepted into the NIH dabrafenib trial if they qualify based on all the other criteria.]

He has recently had a skin lesion found on his ear that is a melanoma. One member had skin lesions, too; but they are side effects of V. And one member told us that she has a number of skin lesions on her arms and legs, but they are all histiocytes.