

## **Summary of ECD Global Alliance Internet Chat**

### **15 April 2017**

7 Attendees

- A member, who is in remission on vemurafenib (V), has just returned home from Mayo after going to see his ophthalmologist and haematologist (Dr. Habermann). He told us he is doing "great". He is now only taking one pill of V a day, with no side effects whatsoever.
- Another member is due to go to Mayo in a couple of months for his check-up. He sees Dr. Go. He has been taking dabrafenib (D), but has been off this for a couple of weeks because of side effects. He has had hand and foot issues - swelling. Dabrafenib also causes headaches. He took V before D (2 V twice a day), and had the same problems, although they are lessened on the D. He said that the V gives harsher side effects – especially the skin rash. It also results in hand/foot syndrome. Dabrafenib has only given him the hand/foot problems. He thinks that D is right for him, but he would have stayed on V if he could have.
- The member on D said that he might talk to Dr. Go about a reduction in dose. He thinks that it all depends on what the PET scans shows in June. Lesser doses of V are needed, because of the difference in half-lives between V and D. Vemurafenib has a half-life of about three days, whereas D's is about 8 hours. So you don't need that much V to get the same efficacy as you get from a dose of D.
- Blue Cross Blue Shield (BCBS) refused to pay for the member's second PET scan, to check on progress of the BRAF inhibitor medicine. He is now on his third appeal. The denial was because the BRAF inhibitor is an "experimental drug - not necessary for long-term health". Mayo is helping him to appeal. He said that "the doctor that evaluated the procedure is clueless about ECD". So BCBS is allowing the use of D, but doesn't allow for a second PET to check on progress. When he was on V, BCBS approved the first PET scan before treatment, and then denied the second one after three months of V (a PET scan costs >\$7000!).
- It was said that, in general, if you are on a clinical trial they don't restrict your scans as much. Once you're out of the trial, they are more stringent. The member will try to get pre-approval for the PET in June.
- A member in remission after a course of V said that he is not having any scans for now, because he doesn't feel the need for them. For three years, no changes have been made in his treatment solely because of any of the scans. After talking to Dr. Habermann, they have decided to stay with one pill of V, as he doesn't see any advantage in switching to D.

On the D, he would need two doses a day because of the shorter half-life. It's more convenient to just take a V just once a day. At diagnosis he had two large masses behind his eyes and his optic nerves were almost severed. Also his eyes were bulging pretty badly. He was going blind in 2014, but has 20/20 vision now, and all his scans and photos are normal. He thinks that this is more important than another PET scan, because this is a clinical result!

- Another member on treatment has had an eye exam. All the tests were OK. The doctor measured for protrusion of the eyeballs and checked the retinal curvature. His eyes had never protruded. He had seen an eye doctor because of unexpected blurring vision.

- A member reminded us that he was diagnosed with ECD 18 months ago (July 2016). The diagnosis was confirmed in Paris. He had ECD in the legs and kidneys, and diabetes insipidus. At the moment he is well, although he does still get episodes of pain. He has received the results of his full body scan. He does not have too much damage, apart from his legs and kidneys. His head and heart are in good condition. He still doesn't know if he is BRAF+. The next tests are scheduled for October, after the ECD Patient & Family Gathering/Congress.
- The wife of a member with ECD told us that he is settling in nicely to his new apartment. He is now in a sheltered accommodation with caring help and adapted furnishings. He saw one of his ECD doctors recently. He is doing well and isn't going back to the doctors for 8 months. He is more active in his new surroundings, and did get sunburned. He is on a dose of two in the morning and two in the evening of V. The doctors are planning for one scan every few years, or as a last resort. They have been able to get the info they wanted by MRI and CT scan, although they are having a problem getting a CT scan of the heart, as the requisition was turned down last October. The ECD doctor got on the case and has put a request in.
- Two members came on after the Chat and apologized that they had missed it. One left a message that she had swapped from V to D because of joint issues, but is now taking a month off chemo because these issues continue. Her eyes have been quite dry, but it is due to the chemo, not the disease. She uses drops and a mask, that she wears in the morning to "start the oil up" in her eyes.