

Summary of ECD Global Alliance Internet Chat

12 Nov 2016

4 Attendees

- We were told by the wife of a member that the tests he had done recently have come back. There are some improvements regarding his "hairy" kidneys. The family notices a big difference when he is not on vemurafenib (V), or when the dosage of V is reduced. He is weaker, has problems swallowing, talking, and moving around. On a dose of two in the morning and 2 in the evening he is doing "pretty good". The doctors opted not to do a PET scan; but they did a MRI and CAT scan to review his lesions. They want him to stay on the V dosage, especially if the lesions are still present.
- A member said that he is having a few problems with his fingers. He is having difficulty holding some objects, but has no pain or synovitis. He said that he has had these symptoms for a long time. He is being treated with Kineret, which he has every day. This will carry on for 6 months under the strict control of the pharmacist. He will have a blood test every month, which goes to the doctor specialising in ECD. The pharmacist must check that he doesn't take too many doses. He has been taking Kineret for 3 months and is still waiting for the "beneficial effects".
He is also a firm believer in the use of plants and plant extracts to help the body. He takes Chlorella, an algae in the form of tablets, which helps to remove heavy metals. He thinks that it is essential to get omega 3 into the diet. Omega 3 is found in flaxseed, rapeseed and fish oils.
- One member is taking dabrafenib (D). He was swapped onto this from V, because V was causing his liver tests to go awry. He is taking 3+3 a day. The doctors are talking about reducing this once he has had a further PET scan. He doesn't like to be exposed to "all of this radiation". He is not having any side effects, even sun sensitivity. He has just had some hair loss "in various areas" [thats enough information!]. He is having monthly blood work done locally, which has shown good results.
- A member expressed concern about why it is taking so long for V to be approved by the FDA. He thinks that the current situation is "ridiculous"! He said that many new patients could benefit from the drug. If it's taking this long with V, what will happen with getting approval for D, trametinib, kineret, etc.? You can get authorization, but the doctors have to be your advocate to get any of these drugs. He said that not every doctor is like Dr. Diamond. Most don't know enough or bother to help patients get beyond interferon.
 - [Editor's note: The ECD Referral Care Center doctors are aware of the newer treatments for ECD. Most, if not all, work very hard to get the best treatment available for their patients. Obtaining FDA approval for an already available medication to treat another disease can be costly and time-consuming. This requires the filing of an application with scientific results from clinical trials showing the efficacy of the drug. The time and money required for this to happen is great. Yet, it all begins with patients enrolling in clinical trials. We are hoping that someday the trials currently open to ECD patients will result in FDA approval for the use of these treatments for ECD.]
- A Canadian member said that there is the same issue with drug approval there. V is not covered for ECD by the provincial plan, as this is not the recommended use for the medication. There is some movement at the provincial level to get recognition in general for rare diseases that need unusual medicines.

- Cholesterol was mentioned. A member said that his overall cholesterol is 157. His "bad" cholesterol is good, but his "good" cholesterol isn't too good. He knows that 157 is a pretty good figure. He was referred on to:

http://www.heart.org/HEARTORG/Conditions/Cholesterol/AboutCholesterol/Good-vs-Bad-Cholesterol_UCM_305561_Article.jsp#.WCeBDdArLnA

A member told us of a woman whose husband had bad cholesterol figures, and so she made a specific muffin to try to help him. She thinks that this was a no-egg recipe. Egg whites are OK, but the yolks are full of cholesterol.