

Summary of ECD Global Alliance Internet Chat

05 Mar 2016

8 Attendees

- A new member came on and just left a message. She was recently diagnosed with ECD and has yet to receive treatment of any kind.
- A member who has been on Vemurafenib (V) for a while, has had to stop it because his liver enzymes rose. He had to stop it for 3 weeks. Now, the liver enzymes are almost back to normal. He has his bloodwork done every other week. He was supposed to start dabrafenib a week or so ago, but his doctor wants him to continue V, taking it two weeks on, and then two weeks off.
- The member who has been having recurrent problems with macular edema has the problem back again, having been edema free for 3 months. She is back on a high dose of steroids. The edema is actually her biggest problem. It seemed that after being on the Sorafenib (she is BRAF-), and low steroids for a long time, the edema was getting better and then "BAM"! Edema is fluid buildup in the macula, which is the center of the retina (and produces the most detailed sight). It affects her vision, making it blurred.

The drug that this member takes, Sorafenib, can also cause liver enzymes to go up. She is having a PET and an MRI at the end of the month. She is taking sorafenib (because she is BRAF-) and steroids for her eyes. She is pleased that she didn't need surgery on her shoulder and starts a 7 week course of therapy this week.

- A member, who is using Kineret, told us that she was still on it, but the time to reapply for funding is coming up. She will then find out whether anyone is going to help her pay for it. She is due for another PET scan in May. She is on Medicare, but the company making the Kineret has a program that she is hoping to stay on for payment. She has been getting the Kineret free from the drug company's program for the last year. She is willing to pay something towards it, but "can't pay what it costs". People on this program have to reapply each year. It would cost her about \$700 every 4 weeks if the insurance was willing to take it on, but last time they said "no thanks".
If Medicare paid, then they would pay 80% plus and the insurance company would pay 80% of what was left. Her part would be what is left, which would be about \$600 to \$700 every 4 weeks. She is going to apply to the drug company. If they turn her down, she will look into what the "out of pocket" max is this year.
- Another new member came on. She was diagnosed with ECD almost 2 years ago, and recently started a new treatment, after having initially been put on methotrexate 2 yrs. ago. She is BRAF+ and has bone lesions, a mass in her abdomen, joint pain and swelling. She has lesions in her legs, back, kidneys, and thyroid, which they removed. For the most part she feels "OK". She said that she couldn't be in the trial for these drugs because of her thyroid. She has just started trametinib, dabrafenib and mekinist. She has her treatment at the Cleveland Clinic in Ohio. She goes at the end

of the month for a PET scan.

The drugs are trial medicines, although she is not in a trial. She gets her medicines through Novartis. She had to send an application to them with her doctor's prescription. She was asked how she got approval for off-label use without trial data.

She told us that she did have a CT scan after one month which showed very little improvement. But she has now been on them for 3 months, with no side effects, and will have a PET at the end of March. "Hopefully they are working."

- One member said that he had been on V since May 2014, starting with the highest dose, and is now on a dose of 1 in the morning and 1 in the evening. "V has a lot of side effects, but if you can manage them well, it's worth the pain and trouble. He is now in remission, with almost no symptoms from ECD and little side effects from V. He thinks that he will have to remain on it "forever", but at a low dose. At present, nobody has the answer to this question!
- We have one member who has just started treatment with V. His wife came on, and said that he was doing well. "We managed to take him out for the first time on Friday, since starting treatment." He is feeling better with very little in the way of side effects. He has a little knee joint pain, maybe a pimple or two, a red rash, and, of course, sensitivity to sunlight. He is as "hungry as a bear". He is on a dose of 2+2 V. They have been featured in the papers. If you want to see how good they look (!), then this is the link <http://www.yorkregion.com/community-story/6372871-newmarket-man-with-rare-disease-must-battle-system-for-coverage/>. This article was written before he started treatment.
- One member stated, "It looks like a lot of competitive drugs are coming onto the market at the same time. This will mean that Roche/Genentech is under a LOT of pressure to push for V's approval ASAP."
- Some of the side effects of V were discussed. It depends, to some degree, on the person taking it. It can range from a bad rash, to blisters on the hands and feet, hair loss, and sun sensitivity. One member said that he has had amazing results. He started it last June, and his brain lesions were cleared 75% in 3 months. Before treatment he had lesions in his kidneys, adrenal glands, bones, eyes, and brain (to name but a few).