

Summary of ECD Global Alliance Internet Chat

07 Feb 2015

3 Attendees

- Two of the Chatters had both been to Sloan Kettering (SK) on the same day, although they had not met up. They were most likely there at the same time. Next time, they will communicate prior to arrival.
- One of the chatters told us that she had had a good visit. PET scan showed improvement in some areas and stability overall. The PET shows no activity in the orbits yet her eyes continue to deteriorate. She saw her eye doctor while she was there, and he gave her a shot of steroids behind her eye. Her doctor said that the PET scan showed there was improvement. He placed her on Sorafenib, a "cousin" of Vemurafenib (V). She had tested positive for the ARAF mutation, and this particular drug targets the ARAF. She started this week taking only one tablet daily, but has to try to build to 4/day. She is feeling fatigued. She is not in a trial, but her insurance company is paying for the drug. She just pays the co-pay. This drug is used for liver, kidney, and thyroid cancers and is just as expensive as Vemurafenib, both are about \$100,000 a year! It appears that it has a lot of side effects. She is not sure when she will be going back to MSKCC; probably 2-3 months after starting the medication. She has no problem with red or pink eyes, but has distorted blurry vision. She also gets macular oedema.

Previously she was on Anakinra, which took away a lot of her symptoms, including low grade fevers. The doctor stopped the Anakinra, and the fevers returned. Her doctor is trying to save her vision, and thinks that the only way is to target what he thinks is causing the ECD, and that is the ARAF. She is only on one pill a day at the moment as her local doctor wanted to see how she would react to the drug. It's hard to say what the dose will be, since they have never used it for ECD. The maximum dose is 800 mg and she is on 200mg. Probably, after her lab tests this week, he will increase the dose.

- Red or pink eyes had been a problem for another chatter. He found that applying a cold compress to his eyes really helped to relieve any discomfort. He had serious bulging of the eyes, and the optic nerves were swollen to the point of being severed. Vemurafenib (V), at the highest dose, reversed all of this in 1 week. He now has 20/20 vision. The masses behind the orbits are shrinking only slowly. He has been doing OK for a few months now, and the only problem that he still has, is hand and foot syndrome. He is not getting any blisters; just bruises, swelling, and thickening of the skin. In extreme cases of this syndrome, nails may start peeling off. Luckily it is "not that bad for me". This is a known side-effect of V. He has found that icing helps a lot. Sometimes he walked outside to get his dog in -- in bare feet, and this helped a lot. He has no other serious side effects. They check his blood monthly, and are doing very detailed checks on blood counts, minerals, vitamins, etc. He does not get low grade fevers.

He got dramatic improvement of his vision when he was on the highest dose of V- 8 pills a day. He said that it takes a few days for the drug to get into the blood stream, so if you take the highest dose, it would be a few days before you "see" the side effects. And, for the same reason, once you

see the side effects, even if you stop taking the drug, it will be a few or so before you are restored to normal.

The patient reported hearing there are people who are taking vemurafenib every other week, and still seeing the same or similar benefits. He cannot tolerate 8 a day, and so he is taking 6. However, he knows that if he takes 8 a day for 4 days then rests a week, and then goes back to 8, he will see better results. This has been shown for melanoma treatments. He had speculated that he was low in vitamin D and asked them to check his levels. They did and he was right. He is now taking 50,000 IU per week.

- Another Chatter came on, who has been on V for 14 months. She has been doing well, although she did have some joint pain in the last week. She now takes 3 V a day. She felt that her walking got worse when she only took 2, but there were no changes on MRI. She cannot tolerate 4. She was asked whether she was troubled by the hand/foot syndrome. She is not, and she goes to the podiatrist every couple of months.
- It was reported by a patient that he heard at MSKCC they are looking into the option of letting patients who are in "remission" come off the drug, but not off the trial. If there is a flare-up of disease, they can restart the V. Maybe, 50% of patients will not have to stay on it for good. The definition of remission is that there is no activity seen on the scans.
- One comment was that "Maybe just a break would be good for us, we could get some sun". The patient starting on Sorafenib was told that, if it works in the same way as V, she should get similar benefits.