

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

### **9 May 2015**

6 Attendees

- A member who has been ill for a few weeks, said he would be missing the Chat because he was going out with his wife for the first time after a long period of illness.
- A member who has had a good result from vemurafenib (V) treatment is now down to 1 pill in the morning and 1 in the evening (1+1). She told us that she had lots more energy and less pain. She has been sent a letter saying that she may be scheduled for further scans about Sep/Oct to see how effective the low dose has been. She wants to go to the conference and doesn't want the times to clash!
- Another member who has been on V is getting off of the clinical trial due to a pending relocation. He is doing well, but knows that he will need something for maintenance. Ideally, he would like to try, or experiment, with something that doesn't have sun sensitivity. "I really miss the sun!"
- The member on the low dose of V misses the sun, too. She can tolerate 30 min in direct sunlight using SPF 50. She was also featured in a magazine article recently. This has not appeared on the internet yet, only in the paper magazine. It concentrates mostly on pictures. A picture of her outdoors with her walking sticks; one when she was 10 doing gymnastics in the yard; another one with her boyfriend on a mountain top that they climbed this Easter; and one with her lifting a weight with her personal trainer.
- A member came on who uses a voice recognition program (Dragon Dictate), to allow him to talk to the PC instead of type. It has produced some "odd results"! He has been on V for a while and said that he was "doing pretty good". He is not on a trial but gets his treatment through the VA.
- The member, who is relocating to San Diego, is hoping to see Dr. Kurzrock in June to "discuss life" after being on the clinical trial of V. He will be keeping us posted because, based on his personal communications with patients on the V trial, most people are afraid of coming off the trial, even though they are in "remission". He is lucky that he is moving to San Diego and Dr. Kurzrock just happens to be there, and has just started taking new patients! He doesn't think that his present doctors are happy that he is dropping off of the trial, although they can still report his results. He has been on the trial for one year. However, he thinks that the trial is not considered "complete" without the remission patients going through the "LOVE" phase. He is one of the patients who has seen remarkable clinical benefits from the V. He would like to find a good maintenance regime for patents that are in "remission". He is hoping that Dr. Kurzrock will be able to answer this question. Dr. Haroche is doing the LOVE trial to try to determine how to cease treatment with V. His latest paper mentions the use of 1+1 doses for 9 months with no relapses.
- A member told us that he had started V two months ago. He is on 4+4. He asked how long it had taken for the other member to get to one plus one.

Someone answered that it took more than a year. She thinks that she had treatment for 14 months, but only took 1+2. She had too much pain in her body when she started on 2+2. It took a week to start working.

The new V starter has ECD in his long bones, in his chest cavity, abdomen cavity, some in his brainstem, and in the area of the pituitary gland. Since his disease is widespread it was said that he would be able to "tolerate" the higher dose for a while.

- A member, who is also in a V trial, told us that one of the most advanced patients on the trial was taking 4+4 for over a year before dropping to 3+3. "You have more areas and surfaces to take up the V." She is doing great now!
- The member who has newly started the V said that he was "doing pretty good" on the 4+4, although he still has bone and joint pain in different areas at different times. It's in his feet at present. The real problem is the sun, his skin heats up really fast and feels like it's on fire, so he ducks inside and out quickly. His face is red for quite a while. He wears long-sleeved shirts and a hat.
- Patients report using much lower doses and/or taking it intermittently (breaks every other week, etc.), and still see significant efficacy.
- A member told us that he was on 4+4 for 4-5 days, and literally couldn't walk or use his hands, even after stopping for a week. The half-life of V is many days, so it takes a while to reach a steady concentration in the blood; and it also takes a while to drop, even after you stop.
- Kathy told us that she has been busy at work recently, and is planning a 65th wedding anniversary party for her parents!
- Members on V discussed sun sensitivity which they all had to different degrees. One had got burnt a few times, and said that you need to avoid a "clear sky on a sunny day". His face and lips were burnt pretty badly just a few weeks ago, even though he was under an umbrella with sun screen on. The lips and head (if you don't have much hair) are most vulnerable to the sun. He has lost his eyebrows due to the treatment. He didn't have much hair to begin with, now he has even less.
- The member who is now on a reduced dose has sun sensitivity and does not sun bathe. But she thinks that she seems to tolerate the sun a bit more now she is on a lower dose. She has had no major burns. Her hair thinned a lot but is now "Super curly, almost like Shirley Temple".
- A member, who has newly started V, said that he had had a lot of hair on his head when he started. In two months, it has thinned quite a bit and when the hair is dry you can see his scalp pretty easily. He thinks that he has a little more energy since starting V. Another V patient said that since the new V starter has a lot of areas to take care of, it will take time but it will happen. As regards his own case, he also had extensive involvement but not too advanced. He has seen "improvements across the board!"