

## Summary of ECD Global Alliance Internet Chat on 9 Nov 2013

21 Attendees

- A lot of the Chat was about the ECD conference held in San Diego on November 1-2, 2013. All the comments were positive, and everyone is very grateful to the volunteers for all the hard work that were expended. Members had also enjoyed being able to “put names to faces”. Members were very appreciative of the fact that so many researchers, with an interest in ECD, had got together. The "official" proceedings from the Medical Symposium will be documented by the researchers, and published in a Medical Journal, so all doctors will have access. When that is published, everyone in the organization will get to see it. It will take a while to get the paper written, agreed upon, and published. But, the good thing is that it is being done, and, by writing it together, they will continue to exchange ideas!
- A few of the messages that were given were -
  - The BRAF inhibitor trials are showing very good results. There are side effects from these drugs, and no one yet knows what the long term might hold for this treatment, but the early results are very promising.
  - If an ECD patient tests negative for the BRAF mutation, they should consider being retested, at an institution with expertise in testing for the mutation in ECD patients. It is looking like there may be a number of people who originally tested negative for this mutation, but when using more sensitive testing methods are found to actually be positive for the mutation.
  - If a patient is being test for the BRAF mutation, it is strongly encouraged that the institution performing the test contact one of the ECD-knowledgeable physicians to ensure the testing process will be done with the level of sensitivity needed for ECD patients. (Please email [support@erdheim-chester.org](mailto:support@erdheim-chester.org) for more information.)

- If someone is on a treatment other than the BRAF inhibitor, and doing well, they should strongly consider staying on the drug that is working for them.
- If you or your loved one is considering trying a BRAF-inhibitor, it is strongly encouraged to enter one of the trials ongoing in the US and Europe if at all possible. The advantages of entering into a trial is that the drug is paid for by the pharmaceutical company, patients are closely monitored for adverse side effects, and everyone will benefit by having the results shared. It is only through clinical trials that treatments can become “approved” which will allow/force insurance companies to pick up the costs of the treatment in the future.
- Three ECD videos have been posted on You-Tube, and can be viewed.
  - 30 second video at - <http://youtube.com/watch?v= ZGZ3CxyO M>
  - 2 minute video at - <http://youtube.com/watch?v=djaRED7UyYg>
  - 20 minute video at - <http://youtube.com/watch?v=HJcSPr4gGTI>
- Kathy hopes that more people will register with the Global Alliance, so that we can show all the researchers, that there are patients “out there”, and that those patients are very grateful for their work, and are willing to help, by joining studies to find better treatment options. To join the ECD Global Alliance, please email [support@erdheim-chester.org](mailto:support@erdheim-chester.org) and provide your name, email address, and state whether you are a patient or a loved one of a patient.
- Members gave some details of their travel “experiences”. Things had gone fairly well for most. We were also told that the food at the conference had been wonderful! (some would say that this is the most important part of any meeting!) One of those who had been in San Diego, wanted to thank Christina and Cindy for making sure that he got back to his room after his leg gave out on him again. His thanks will be passed on.
- One member took her primary physician with her to San Diego. The doctor was feverishly taking notes during the nutrition and exercise presentations.

And now she is challenging the member to try out all that stuff! It was said that this member must be careful who she takes where!

- A member had told her physical therapist about the conference, and the therapist would like to come, too. This should be possible for next year. Kathy would like to fill all the hotels in Bethesda, with people interested in learning more about ECD!
- Another member of the Alliance has booked to go to the NIH in December. He is expecting/hoping to have a further biopsy taken to test for the BRAF mutation.
- A member told us that her Doc is trying to get her on the BRAF inhibitor, but her insurance has to say OK. Her peginterferon is being increased. A member told us that her liver hadn't liked it when her dose had been upped!
- If anybody wants to buy an ECD t-shirt, or wristband, like those that were on sale at the conference, then they should email [Juliejones2261@gmail.com](mailto:Juliejones2261@gmail.com). The profit goes to the Alliance. One member has already had several people ask about ECD, since he has been wearing his T-shirt.
- A member has been thinking about trying to raise some money, at her workplace for the ECD Alliance, but hasn't started yet. This is the time to start doing fundraisers - while people are still "energized" after the conference. If anyone is interested in doing a fundraiser and needs help, please contact the ECD Global Alliance at [support@erdheim-chester.org](mailto:support@erdheim-chester.org). There are other members in the organization who are willing to help you.
- RuthAnn told us that she has been working on the caregiver issue. She hopes to get out another email in the next few days.
- A member told us that she is due to start vemurafenib in December, but still has no answer from the Norwegian government as to whether it will pay for the drug. All that she has received is a letter saying that, it can take up to 2 months for a decision to be made.
- A member is starting vemurafenib in January. He told us that his Doc intends to keep him on Kineret too, and he was advised to ask the Docs at the NIH,

and/or at Sloan-Kettering, what they think about taking vemurafenib and Kineret at the same time.

- A member is going to the hospital, this week, for her regular check, and also to see about her shoulder, back and depression. She thinks that she is “doing pretty well”, but was having back pains, and has, recently, fallen twice while bending down.
- A member has started on an anti inflammatory drug this week (indomethacin/ Indocin) along with his interferon. His doc would prefer him to be taking meloxicam. This has less side-effects than indocin, which “has a tendency to eat holes in the stomach lining”. It has really helped with his leg pain. Unfortunately, he will have to stop it, for a bit, since he has another biopsy next week to confirm his BRAF status.

He had a bone scan last week, and this showed that the active areas have progressed. There is spread in his legs and arms. He thinks that he will, probably, be a candidate for vemurafenib treatment.

- A member came on who lives on a Greek Island. The weather is so good that she is still able to swim (the only exercise that she can manage, as leg pain is bad at the moment). She is being troubled by terrible sweats, and heating up. She is also feeling faint, and very wobbly. She wonders whether the drugs that she is taking (methotrexate and steroids) don't go together. She was told that some ECD patients do have sweats, especially night sweats. She was asked whether her BRAF status has been tested for, and she has tested positive. She sees the same Doc, in the UK, that another member also sees. The Doc wasn't keen to put her on vemurafenib yet. The clinical trial for vemurafenib has locations in England. The sites listed include Aberdeen, AB9 2ZB, London, SW3 5PT, Sutton, SM2 5PT. These are the locations that are participating in the trial, but we would have to make certain that the doctors at those sites would accept an ECD patient.
- It was said that it was a good thing that the conference won't be in sunny San Diego next year, since a lot of members will be on vemurafenib, and shouldn't be in direct sunlight. There is a incidence of skin cancer as a side effect of the vemurafenib treatment. Not the melanoma form of cancer, but the kind that is, usually, easily excised. This would be a big problem for the member who spends time at her Greek home. One member lives in Las Vegas, and is also

exposed to a lot of sun. Another hopes to get into the vemurafenib trial. It is very sunny where he lives, and he likes to spend a lot of time outside. On this topic a member said that he would not avoid vemurafenib because of the sunlight concern. The results, in the trials, have been dramatic, and neither the French nor U.S. doctors have spoken about sunlight being an issue. However, close follow ups to ensure there is not a problem would be required.

- A member has been recently using Facebook to Chat with a female ECD patient. She has been put on Remicade. The co-payment for Kineret was too high. This is the first thing that they have given her. She is unable to work, and is being denied disability. The member has told her about the Alliance, this chat, and the fact that ECD is a fast track disease for compassionate allowances. He is hoping that she can put the pieces together from there.
- Someone else, on the Chat, has a friend who took Remicade for ECD. He also took methotrexate. He is still alive(!), and is now taking Cimzia.
- A member, who has had previous falls, has fallen again, “messing up” his right arm. He said that it was “weird” how he fell. Physiotherapy hasn’t helped, and a cane would also be no help, either. He told us that, “Things just go off kilter in my head”. He wants to speak to his Doc, and has left a message. No response so far.
- A member told us that her walking sticks sometimes “get in the way”, and don't always help her to stay on her feet.
- Members said that they were amazed at how many different approaches there are to ECD. When one member had been diagnosed, back in 2001, there was no known treatment, and just living seemed to be the immediate goal. Now, so much progress has been made, that just living isn’t enough! Dr. Estrada-Veras said that treatments are keeping patients stable. They are not a cure, but they are giving the doctors time to find a cure.
- At the conference next year, we will be hearing how Vemurafenib has been working, and getting an update on its side effects for people with ECD. It was said that it would be great to see everyone running around next year!

- It was suggested that, if you use FaceBook, you should stop by the ECD Alliance FB page and leave a comment, question, or word of encouragement. Those that went to the conference could leave a note about what they had got out of it. This will help those people who find the Global Alliance on FB, after being diagnosed. (Editor's note: Please note that the FB page is not "owned" by the ECD Global Alliance, although it is a great place to connect with others in the ECD Community. The [www.rareconnect.org](http://www.rareconnect.org) is also a good place to connect with others and share questions and answers.)
- Welfare payments were discussed. It was commented that the reward for having a little cash on hand is to have it deducted from the monthly benefit total. One member has had great difficulties. His youngest son has cerebral palsy. Getting the financial help that is needed has been very difficult. And now, he, himself, has ECD and needs help!
- A member and his wife are to be congratulated. They have just celebrated their 20th anniversary together.
- One member told us that he believes that he still has some active disease in his bones and eyes. He tends to get double vision at the extremes of gaze. He told us that he had had steroid injections right under the eye ball, into the socket. This helped to shrink the tumor that was around the optic nerve. He said that "Watching someone coming to put a needle in your eye is uncomfortable." [Summarizer's note; I'll bet it is!!]