Summary of ECD Global Alliance Internet Chat 17Aug2013

9 Attendees (a truly Global gathering! Members from the UK, Belgium, Israel, the US, and Australia were present!)

- A member, from Israel, who has not been on the Chat for a long time, came on. His wife has ECD, and has just had a 3 month course of vemurafinib. She had improvements in speech within 2 weeks, and with balance in a month. She also feels much better. Side effects are confined to a rash all over her body. This is treated with cortisone creams. This drug (vemurafinib) is a BRAF inhibitor. The member said that he thought that it was most important for us to be tested for the BRAF mutation.

- A member asked about the treatment of people with the BRAF mutation. This depends upon whether you are "ill". The doctors, probably, won't change your treatment if you are stable. Vemurafinib is a new drug, and is being trialed at the moment. It was developed for patients with malignant melanoma, who also have the BRAF mutation. A lot of ECD patients have the BRAF mutation too, and that is why it is being trialed.

- A new member came on, who is from the Las Vegas area. He was diagnosed with ECD a month and a half ago, after 2 years of searching for a diagnosis. He has just started his second week of interferon treatment. He doesn't feel as bad as he expected to feel. He had a short bout of blurry vision, for a couple of hours, but it went away and he feels "flu like" and very tired. He has been being treated for Diabetes Insipidus for more than a year. He is a single father with two children aged 17 and 12. The younger has special needs.

  He has been trying to get to see Dr Kurzrock. He has a contact number for her at UC San Diego, and is waiting for a response. He has been given some information about another ECD patient in Las Vegas, and has sent an e-mail, but is yet hear back.

- A member told us about an unusual experience that she had this week. Her insurance carrier, which handles Medicare for her, sent a doctor to check her over, just in case her own doctor was missing something. At first, the member thought that this was unnecessary, and a waste of time. Towards the end, the member said that "There is one more thing you probably should know about my health: Erdheim-Chester Disease." When the member mentioned "foamy histiocytes", the doctor immediately took notice. She had been a hematologist in Ft.Worth, and had heard of this! After that, she asked the member, about four times, if she had any kidney involvement. So far, the disease has primarily affected the member's lungs. The doctor must have run into someone with ECD, with kidney involvement.

- A member asked what to expect with interferon treatment? Members said that interferon makes most patients feel very tired and ill (like having the 'flu). Many patients, who get the aches and fatigue of interferon, begin to feel better eventually, but it takes months.

- A member, who has been at Sloane-Kettering recently, is back home and has had four days on vemurafinib. So far there has been no “miracle cure”, and no side effects. The doctor, at Sloane Kettering, said that 40% of patients get a rash. This is not itchy or painful, but you do have to be careful of direct sun.

- A member was interested to know more about this rash. This member has red spots all over, but no itching, and has had more that 20 biopsies. None of these have ever showed anything. The spots came along 10 years before the ECD diagnosis.

- Interferon was discussed. We were reminded that the pegylated form of interferon reduces the number of injections from 3 a week, down to 1 per week for most patients.

- Different countries have different systems regarding payment for interferon.

  In the UK, the National Health Service pays for all drugs, provided they have been “approved” for use.

  In the US, some insurance companies will pay for interferon without any problems, but other companies require the patient to fight for it. One member told us that he had had to fight for over a month, before he got his interferon treatment approved. If it had not been approved, he would not be getting any treatment at all!

  There are a couple of cases, where patients are still trying to get the insurance to pay. In some cases, the pharmaceutical company is helping to pay the costs. It is very confusing. The problem, in the US, is that there are no formally approved treatments for ECD. All treatments are considered "off label." This means that each case can be viewed independently. It would be different if there was an "approved" treatment that had been clinically shown to help patients. It is one of the things the ECD-GA would like to make happen, and why clinical trials are so important. In the US, the drug manufacturer will sometimes "Help" pay for the cost of the interferon, if the insurance company won’t pick up the cost, and it is too much of a burden on the patient. This is usually done under a program called "patient assistance" or “prescription assistance”. The ECD-GA website has links to some organizations that will help patients qualify for prescription assistance in the US –

  https://www.pparx.org/Intro.php
  http://www.rxassist.org/
  http://www.togetherrxaccess.com/

In Belgium interferon is not paid for by insurance. The maker of interferon, will pay for 3 months of treatment, but then the patients have to pay for it themselves.

- Some new medications, that are being used, or being considered for use, were discussed. A member remarked that it was “amazing how many more drugs are getting used for ECD. Wonderful that doctors are trying them out with patients and having some good effects”.

  Fampyra is being used by one patient, from Norway. She has said that, soon after she began taking it, she had felt that her limbs were less "tight". She has remained on it, and is finding it helpful. She has not mentioned any negative side-effects (She is on holiday, living it up on a Greek Island, at the moment!).

  Dabrafenib, another BRAF inhibitor drug, is said to have less side effects than vemurafinib. No ECD patient is known to be on it, yet.

- A member who has been troubled with back pain told us that she is doing much better, but still needs “some more improvement”. She thinks that things would have gone faster, if she hadn't developed asthma on top of her ECD lung problem!

- Members that are going to San Diego said that they were looking forward to meeting up. One member said that it was a pity that she, and her husband, did not understand English very well, and so San Diego' was “not for us”. Kathy said that if they could make it, the GA could TRY to find an interpreter.

- Members then told the Chat where they came from, and whether they were ECD sufferers or carers; one patient was from the UK, and three from the US. There was an Israeli whose wife has ECD, and a Belgian whose husband is the patient. There was one member whose husband had died from ECD, but the diagnosis was not made until after his death.