

Summary of ECD Global Alliance Internet Chat

6 Feb 2016

10 Attendees

- A member has been checking for side-effects of Vemurafenib (V) at <http://www.drugs.com/sfx/vemurafenib-sid...> and noticed that there is a food side effect with it; V may increase the blood levels and effects of caffeine.
- A member whose husband is about to start V asked for information from those who had experience of it, particularly regarding what to watch out for "apart from the sun". She was advised to be aware of the possibility of joint pain, skin rashes, and hair loss. "Relaxation, workouts and shopping, but no work and no sunbathing!"
- The wife of the member starting V said that they had got the OK from the insurance and hospital to get V for him. They are just waiting for his doctors to set it all going. This is at the Princess Margaret Cancer Centre in Toronto. They have had the services of a "fantastic" social worker who has worked well with the insurance company. They are still awaiting the final details, but assume that they are looking at an upfront cost of 12,000 Canadian dollars. They think that they have this covered having raised it from the family, etc. Manulife will reimburse them.
This is all being done through her father's business insurance plan. But there are only 3 employees in his company, so the premium will rise. They will have to wait to see how much it goes up. For now he will be taken care of. In Canada, the people pay a large amount of tax but the health system does not cover those with Rare Diseases.
- Rare Disease day was mentioned. There is a Rare Disease day in the US and worldwide at the end of February. See these websites for more information <http://www.rarediseaseday.org/>, <https://www.raredisorders.ca/>, <http://www.rarediseaseday.org/events/world>, <http://treatrareisease.ca/>.
This is one of the reasons why this couple is going to start sharing his story with the media in the next week. It is expected that in the following days there will be some political action to get relief for rare diseases on a whole, although this will increase everybody's health insurance premiums.
- Another Canadian member will soon find out more about his doctor's plans. The other Canadian said that they could perhaps join forces in getting the V treatment for both of them. It would mean having to go to Princess Margaret on a regular basis to pick up the V.
- A member who has had V treatment said that she was supposed to have a check-up in February, but nothing is set up at the hospital before Easter. She thinks that the doctors don't think that the earlier follow-up is needed. She feels fine, and is anxious to hear if she could have a break from the V.
- Another member on V was on a dose of 4 in the morning and 4 in the evening. Then they

dropped it to 2+2 a couple months ago, and raised it again the previous week to 3+3. So far he is "going well" on this dose.

- Two European members told us that neither of them have to pay directly for their healthcare. It is all paid for out of taxation. The member from Norway has to pay for the flights to Paris where she is being treated! Treatment is also covered to a certain extent, when these members are treated in other European Union countries.
- The Canadian members talked about Trillium (Trillium is a Canadian provincial program that will help to co-pay for medicine if private insurance company refuses to cover it.). One of them had signed up with them when he had his kidney transplant.
- We were told that typing is second nature to one of the Chatters. She was born with severe hearing difficulties. Using TDD - TTY (Telephone Device for the Deaf), she used to spend hours and hours talking on the phone and the rest of her family were begging her to get off. It came to the point that the family got a second phone!
- The other Canadian on the Chat is in remission. He said that his doctors seemed to get a bit more interested in his case when it appeared that the ECD was affecting his "new" kidney, the one that had been transplanted into him.
- A new Chatter came on. Her husband has ECD and they live in Pennsylvania. He is going to Sloan Kettering to see Dr. Diamond, and just started on a new drug trial. Since he's not BRAF positive, there weren't too many options, especially since their insurance wouldn't cover Kineret due to Medicare definitions.

This is very frustrating as it often affects those with rare diseases like ECD. They are going to contact the doctor whom diagnosed ECD in another member, to see if he will consider starting an ECD Patient Care Center. It was suggested that they should join the Rare Disease movement and help to voice this.

They have been in touch with Justice in Aging and Medicare Rights. Both groups thought that the denial of Medicare approval was wrong, but their appeals continue to be denied. The following site may be helpful <https://www.medicare.gov/claims-and-appe...>

They are now on their 4th appeal! They have heard of NORD and plan to get more info from them.

It was said that if there is an issue with cancer, then approval cannot be denied. At the moment, it can be argued that ECD is a cross between inflammation and cancer, but ECD has not yet been classified as a cancer, or at least not in the Medicare world.
- On the CORD petition it states that "Canada cannot continue to treat rare diseases one at a time. With more than 7,000 rare diseases, Canada needs a comprehensive approach." A member commented that "We are not rare people, just have rare diseases." We need to advocate changes in the Medicare statute, to allow people with rare diseases equal access to Medicare coverage. A member always talks about ECD when she goes to a doctor appointment. She wore her ECD awareness t-shirt when visiting the hospital and used it to start a conversation.
- It was also suggested that if they have a friend who is a lawyer they should get him/her to do some calling. One member did this last year when Blue Cross Blue Shield (BC/BS) wouldn't

approve a PET scan. She doesn't know what the lawyer said to them, but they don't fuss at her any longer!

- The effects of V on members' weight was mentioned. One said that although she eats a lot, she doesn't lose weight either, although she is not actually overweight. Another member said that he has lost about twenty pounds while on V. It changed the taste of everything, so he didn't eat much, and so far he has not gained any weight at all. He was on 4+4, and now is on 3+3.
- A member came on who has not been able to chat for a while. He has moved from Houston to Traverse City in Michigan. This is in Northern Michigan, the "upper lower peninsula" is what the locals say.
He asked whether anyone had experience in getting V without being on a clinical trial. This is the question that is being raised in Canada, too.
- On the previous week's Chat, there had been some discussion about getting V off label. It can be done but it depends on which insurance you have.
- The member who has moved to Michigan remains on a trial because it will cost less to fly to Houston once a month, than it will cost to get the V as a prescription drug. He has been taking V for several years now. He has no idea of how long the trial will last. He has had good success with V although he had many side effects at first, skin lesions and hand foot syndrome. These were fewer when the dose was reduced. He started with 4 + 4, but is now on 2 + 2. He thinks that V is one of the best medicines for those of who are BRAF positive. He has been told that he is now stable. A nurse told him "listen, honey, around here no one dies from stable"!!
He could feel the improvement from taking it within a couple of weeks. He will need to fly to Houston for a follow up once a month or so.
Transportation and costs are much less than his co-pay through Medicare, which was about \$2000 per month! He thinks that the total cost right now is about twice that much. He supposes that the cost of V will go down sometime.
He had had ECD for about two years before being started on V, and had started on anakinra (Kineret) and did well on that. He thinks that he was very lucky because he was diagnosed early. When he was investigated they found just one mass, but this was on his heart. He had some shortness of breath (just a "mild" problem for a trumpet player in the Houston Symphony Orchestra!!). Then he developed an atrial flutter (a disorder of the rhythm of the heart). He had that treated, and then had a pacemaker fitted and is doing well.
His cardiologist had ordered a cardiac MRI. He took the images to a "tumor board", a group of cardiologists from all 7 hospitals at Texas Medical Center (TMC), who can share their knowledge and opinions. One of the doctors walked up to the cardiologist and informed him that MD Anderson has doctors who know about this disease! He had the pacemaker fitted after the ECD diagnosis. They did not remove the mass from the heart and one of his monthly checks is a cardiac MRI. The mass has been "very, very slowly" shrinking. He had a heart attack a long time ago and takes plavix and aspirin. The heart attack was not related to ECD. He doesn't take any other heart medicines.
- A few members spoke about the Paris conference in the Fall. Some said that they would definitely be there, and others would be there if they could.