



**CDKL5 ALLIANCE MEETING MINUTES
CDKL5 FORUM BOSTON
November 5th, 2019**

CDKL5 Alliance Meeting was held at the Royal Soneesta Boston Hotel, Tuesday the 5th day of November, 2019 at 12:57 pm EST.

Members Present:

Lynn Jafar – LouLou Foundation
Ana Minogrance– LouLou Foundation
Carol-Anne Partridge – CDKL5 UK
Amanda Jaska - IFCR
Rick Upp - IFCR
Sangeeta Staley – CDKL5 Canada
Marija Kalabic – CDKL5 Canada
Natalie Ladly – CDKL5 Canada
Baptiste Sorrentino – CDKL5 France
Penny Howard – Hope4Harper
Dustin Howard – Hope4Harper
Norberto Fischer – CDKL5 Brazil
Katheryn Elibri– CDKL5 Research Collaborative
Sandra Lopez – CDKL5 Spain
Constance Russ-Mohl – CDKL5 Germany
Patrick Benz – CDKL5 Switzerland
Antonino Caridi – CDKL5 Italy
Maria Ivanova – CDKL5 Russia
Paula Connolly – CDKL5 Ireland
Richard Li – CDKL5 China
Jainu Jogani – CDKL5 South Asia

1. WELCOME - CHAIR

Carol-Anne Partidge welcomes the group to the 3rd CDKL5 Alliance meeting held at the 2019 CDKL5 Forum. She assumed the role in June at the CDKL5 Alliance conference in Edinburgh. She reviewed the agenda for the meeting and added that IFCR would like to discuss CDKL5 Connect.

2. MEMBERSHIP UPDATES – ALLIANCE MEMBERS

Connie from CDKL5 Germany announced that they will be hosting the CDKL5 Family Conference in 2021 with CDKL5 Switzerland and CDKL5 Austria. The conference will take place close to the Swiss border in Constance around the date of June 19-20th.

The Spanish association is happy to introduce their CDD support guide in Spanish. They will send the guide to all hospitals in Spain to all the paediatric and adult neurologists. They will also send the guides to genetic laboratories so they can contact CDKL5 Spain when they identify new patients. They also hope they can open the door to all Spanish speaking countries in South America with the goal of having official representation by the end of the month.

Baptiste from France has also started process of creating a CDD support guide.

Roberto from Brazil presented that they now have 30 CDKL5 families identified in Brazil. They are very happy to be included in the international alliance today.

Carol-Anne is working on a letter of intent to recruit a research coordinator in UK.

Richard from China very excited to be here and to announce that they have their own association as of two weeks ago. The workshop in Tokyo inspired them to create their own association. They set up a meeting in October and invited 5 clinicians in China and had 38 patient families represented. They plan to set up a clinical research centre in China by the end of 2020 and would like to have a forum set up in China.

Maria from Russia plans to organize the first parent meeting in Moscow next year.

Antonio from Italy announced their main activity is on the Italian registry which was completed last year and now are trying to fill the database. Their new objective is to see how this information can be accessed by researchers and pharma. They are putting procedures in place to let them know they have that information. They are hoping that they will be able to open the registry to the rest of the countries included in the EU. They are also discussing design elements in the trials and they believe that the alliance should be who pharma contacts if they are interested in running a clinical trial (entry point for pharma and industries for gathering information) and that the alliance should support those who are working on developing the CDKL5 scale. We need to demonstrate to pharma and industry that we are doing our part.

Paula from Ireland announced that they now have 10 families diagnosed with CDKL5 in Ireland and she believe there are more there. She was also happy to announce that CDLL5 Ireland is now participating in research at Trinity College in Dublin.

Saneeta from CDKL5 Canada said that they have two CDKL5 clinics in Canada. They are struggling because families aren't using them and the few families who are aren't gaining value due to low numbers. They intended to meet up with the Denver clinic to gain some insight into how their clinics run in hopes of creating some continuity across all clinics and to help improve their clinics. They will be hosting the family conference in 2022 in Toronto, and they funded their first research project with Dr. Eubanks and hope that it will shed new insight into CDKL5.

IFCR has hired a statistician and can connect us with them to collect similar endpoints in gathering information. Heidi is the point of contact. IFCR has a series of guides available

online and they are happy to share content for any countries who would like a copy. Please contact Rick directly for the original files for translation.

The CDKL5 Research Collaborative is raising awareness in the state of Michigan and in the midwest. They are also in the process of funding their first research project. They participated in rare disease day locally and at the state level.

CDKL5 South Asia has now identified 12 families and received the stamp of approval from India in March. They are focusing on identifying children who potentially have CDKL5 and connecting them with the right resources and to have them ask the right questions in order to be correctly diagnosed. They are working towards having a clinical trial available and creating a clinical centre focused on CDKL5 with a therapy driven approach. They are considering combining with the middle east to have more success. They are going to connect with Carol-Anne who has identified some families in SA.

3. ALLIANCE PRIORITIES 2020 - CHAIR

- a. Community Advisory Board
 - a. Formal structure for pharma/researchers to be able to work with the Alliance to create clinical trials and creating meaningful endpoints. Many of the clinical trials are only in the US so members of this board would need to work to understand the regulations in their respective country in order to make meaningful contributions to pharma. They also need to decide/define what symptoms they want to focus on (i.e. sleep, seizures, GI, etc.)
 - b. Sit alongside the alliance but would be people involved within the community, clinician/scientists with information about clinical and preclinical with the understanding of the parent voice. Representatives would be from Europe, North America, Asia, LouLou, and Industry. When people are interested in hosting a CDKL5 trial, they would go through the Director who sends them to the board to ensure that our kids are getting the most out of the trial and that we are targeting the right symptoms, the design of the trial, etc.
 - c. Vote on creation of the Community Advisory Board in principle – vote result: yes
- b. CDKL5 Partnership Director
 - a. There is no consistency for the Alliance when the Chair is rotating. Carol-Anne suggests that a partnership director role is created for continuity within the Alliance and projects that has started. Carol-Anne has offered to take on that role and is thinking it is a 3 year term. This role would include engaging with the community, feeding information back to the chair, attending events, being the focal point for the alliance. The website also needs to be vastly developed but it is difficult to complete ongoing projects and coordination in the rotating chair position. The group agreed that a job description needs to be created to ensure there is no overlap with the chair.
 - b. Carol-Anne to send this job description to the Alliance for review with action items for the role.
 - c. Voting on creation of a Partnership Director in principle – vote result: yes

- c. Symptom checks online
 - a. This would be in addition to the clinical care guides available on the website. Ex. If children are crying for days on end it could be constipation and here is what you can try. We need to make it standardized and consistent for all CDKL5 Symptoms.

4. MEMBERSHIP BENEFITS AND REQUIREMENTS

Dan not present.

5. ANY OTHER BUSINESS

IFCR - CDKL5 Connect

We need to have a patient contact registry that is global. Age, sex, where they are being cared for, etc. IFCR has created CDKL5 Connect. It is a database that is highly customizable that they are willing to share so we can better collect information globally. If you have a CDKL5 website in your country that can host a link to CDKL5 Connect, Amanda can add it so that aggregate numbers can be shared but not marketed to. Helen's work will be combined with ODC database. Amanda will share the slides with more detail about CDKL5 Connect so that everyone can take a better look.

6. TERMINATION OF THE MEETING

The meeting was terminated at 1:55pm.

NEXT MEETING DATE: June 26-27 in Houston, TX