

ERN Support and Training programme- WP17- EJP RD

Report Workshop “New Therapeutical Approaches for Inherited Retinal Dystrophies”

Date/Venue/Format

27 and 28 March 2023 at The Príncipe Felipe Research Centre (CIPF), Valencia, Spain. The format was onsite except for one speaker who gave her lecture online by Teams.

Numbers (participants, speakers, ERNs represented, patient representatives)

18 ERN-participants, 10 non-ERN participants, 14 speakers (researchers or clinicians, three of them from the EYE ERN), three speakers as patient representatives. Participants and speakers came from 12 different European countries. Most ERN-participants belonged to EYE ERN but, other ERNs were represented including BOND ERN, EpiCare ERN, ITHACA ERN, MetabERN, and EndoERN. Apart from participants and speakers, seven persons participated as organizers (registration, uploading presentations, assisting speakers and attendees, helping with microphones, etc.).

Analysis of the workshop satisfaction survey

The satisfaction survey reflects attendees found extremely useful the training workshop with an excellent programme and organization. The best aspects that they highlighted were:

- The presentations, the new studies presented and their diversity related to this kind of rare diseases. They commented that it had a multidisciplinary approach of the different therapeutic strategies covering preclinical and clinical aspects with a good connection between research and clinical platform. Well structured programme.
- The workshop helps to establish new scientific contacts.
- The reduced number of attendees that helped them to interact each other, discuss, exchange ideas. They felt comfortable with the rest of participants.
- The presence of patients with their testimonial was crucial to focus the research.
- Being on time and to provide all the information (both scientific and social). Helpful information about public transport, important links and other important information for the foreigners were sent before the start of the event.



- If this workshop could be repeated after some time, please keep the information and personal contacts to exchange them in the future as well and to see how the research and clinical praxis progress.

Regarding ways to improve this kind of workshops:

- Some participants indicated that it should be interesting to include a poster session where young researchers and clinicians show their data looking for advice, for improving interaction or expanding their network.
- Some participants suggested to increase the duration and the number of participants to this kind of workshop.
- Some participants indicated that was fine the way it was organized.

The workshop had six sessions for different therapeutic approaches. All of them were extremely useful for the interviewers. They considered that the time for questions, lectures, etc. were always or almost always adequate. Most of them indicated that the information they learnt be implemented in their practice. All of them considered that the training contents were appropriated for their level of knowledge/expertise (just right).

As example of biased presentation, they provide the following examples:

- To search for new ways to treat their patients.
- To inspire them to work harder to discover new therapies
- To stablish collaborations with other attendees (ERN and non-ERN) and to apply for different projects.
- To find some ideas and advice for design new experiments e.g., with organotypic cultures, iPSCs.
- To know the new clinical trials.
- To take home a good impression of the different options available for therapeutic approaches. To learn about them.
- To offer patients a global perspective of new therapies in development.

2-3 participants' testimonials

Participant 1: Very interesting and dynamic workshop. Very well organized.

Amazing approach. Very fruitful for networking. A great experience.

Participant 2: Very well organized. A good balance between basic and clinical research. It could be improved by including poster session for young researchers and clinicians.

Participant 3: The whole program was really complex with the short breaks enabling to discuss various topics from the lectures and to establish new



scientific contacts. We were well informed about all activities combined with the workshop in time, the program was well structured and cover a variety of scientific and clinical topics. Additionally detailed helpful information about public transport, important links and other important information for the foreigners were sent before the start of the event. I would like to cordially thank to Mrs. Regina Rodrigo and her team, colleagues for a very nice and pleasant workshop, a lot of new information and insights into the problems of RDs. Great! I am not sure, what could be improved... It would be very nice, if such a workshop could be repeated after some time, that the information and personal contacts could be exchanged in the future as well and to see progress in the research and clinical praxis.

If applicable a main result of the workshop (e.g. working group created to do xxxx)

Several participants have networked. They plan to collaborate and apply for different calls. Some participants asked for the possibility to organize a new workshop in the future with more participants.

Conclusions, lessons learned, best practice to keep in mind.

As organizers we believe that participants enjoyed the scientific program, and learnt about new therapies. The workshop provided them a general overview of the latest therapies on IRDs. The speakers gave very interesting talks. Some of them shared their presentations and most of them shared several scientific publications. The presence of patients was very important for the participants. Patients shared their experience after being treated. It was very emotional and encouraging. Our impression is that it was a very rewarding experience for everyone. Members from different ERNs participated specially from EYE ERN. They commented that the information was very useful for their work at hospitals. In general, they were very grateful for the good scientific and social organization as well as for the information they received (training material and social events). In the future it would be good to include a poster session and a slightly larger number of participants as there were people who was not able to attend and were very interested in this workshop.

