

ERN Support and Training programme- WP17- EJP RD

Report Workshop “Challenges and barriers for gene therapies in rare neurological disorders: preparing the next generation of clinicians and researchers”

Date/Venue/Format

Dates: 29 and 30 June 2023

Venue: Hospital Universitari Vall d'Hebron

Format: Hybrid (online and face-to-face).

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

Participants 124

ERNs represented in participants: ERN-RND, Euro-NMD, EpiCare, ERN RARE-LIVER, ERKNet, ERN-RITA, ERN-EYE and MetabERN

Speakers: 28

Patient representatives: 4

Analysis of the workshop satisfaction survey

Most of the respondents considered the quality very good or excellent (35 out of 36). All the respondents said that the workshop was useful or highly useful for their research and/or professional activity. Most respondents considered that the training contents were appropriate for their level of knowledge and expertise. Most of the respondents were also satisfied or very satisfied with date and time, connection process, organization and speakers. Participants that reported less satisfaction in the structure and organization were unhappy due to the density of the program. There are variable preferences for face-to-face or online. Interaction opportunities and Q/A time were satisfactory for most of the respondents.

2-3 participants' testimonials

Testimonial 1: "I recently attended an incredibly thorough program that left me thoroughly impressed. The online option was a real game-changer,



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making it not only convenient but also more practical. Keep the online option; it's very useful and make possible the participation of a lot of people"

Testimonial 2: "The program featured outstanding speakers and highly practical content. The organization was top-notch, and the quality of the speakers was impressive. My only suggestion would be to include Q&A sessions after each talk. Definitely waiting for a second edition"

Testimonial 3: "I had a fantastic experience at the event. The organization was impeccable, and all the speakers were punctual and of the highest caliber. The only minor issue was that the 3-hour sessions, followed by discussions, were a bit too long. Perhaps shorter sessions would be more comfortable."

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

- Commitment to continue with formation in the field
- Person-to-person scientific collaborations between speakers and with and between participants.

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

The workshop brought together a diverse community of clinicians and researchers dedicated to advancing the field. This event addressed the unique hurdles faced in the pursuit of gene therapies for rare neurological disorders, a critical and hot topic in rare disorders.

We were lucky to put together expert speakers, exploring topics such as the latest innovations in research, experiences gained from clinical trials, and the viewpoints of various stakeholders, including regulators, payers, manufacturers, and patient associations. The diversity of perspectives fostered a comprehensive understanding of the field's multifaceted landscape.

Attendees had the opportunity to engage in meaningful discussions, fostering a friendly and collaborative atmosphere. We think we failed in an adequate dissemination of the event, and we think that participation of colleagues from other parts in Europe was poor. We also believe that some of the speakers would have loved to come if they were more aware of the interest of the workshop. This is a first edition experience.

Regarding organization, we can improve the scheduling and the dynamicity of discussions. Even, some engaging activities and previous material will help people to prepare for the more difficult topics.



We are happy to have the support of EJPRD. This is a workshop that cannot be organized without this kind of financial and structural support, and we think that having this kind of multidisciplinary activities could make Europe and European scientists and patients be more ready for the progress to come.

We plan to have a second edition. Trying to see how. We expect that this time it will be even more successful.

