

ERN Support and Training programme- WP17- EJP RD**Report Workshop “Contemporary outcome measures in NeuroMuscular Diseases”****UEMS-EACCME®: LEE22-00038****Date/Venue/Format**

4th and 5th March 2022 – Novotel Nice Centre Vieux-Nice 8-10 Esplanade du Parvis de l'Europe 06300 Nice – On site and by videoconference (teams)

The EJP RD Workshop consisted of both presentations by experts in the field of neuromuscular diseases (NMDs) as well as interactive panel discussions and quiz sessions. An educational tool containing the outcome measures used in the different pathologies discussed has also been made available to all participants.

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

The workshop was followed by 91 persons in total, 52 on site and 39 by videoconference, distributed as follows : 28 speakers, 51 participants, 4 guests (patient representatives, labs and the director of research and innovation in Nice hospital) and 8 members of the staff (Pr Sacconi's team and event agency).

2 patient representatives were present.

In total, more than 60 persons were affiliated to an ERN-Full Member or Affiliated Partner Institution.

Analysis of the workshop satisfaction survey

The workshop was useful or very useful for 97,5% of people. Most of time, people appreciated the high quality and the expertise of the speakers in the different fields as well as the interactive discussions with them. The session about the different clinical outcome measures for myopathies was the most appreciated : 95% of people found this session useful or extremely useful (Votes' results : 50% very useful, 47,5% useful and 2,5% fairly useful).

For most of people, the workshop was well organized and the program was rich and complete. For people present on site, we made available a bag containing the program for the two days, a small notebook and a pen to take notes as well as a document containing a QR code to access the educational tool with all the tables of outcome measures discussed by the experts. Also the participants on line received a link to access this educational tool. For 65% of people who attended the workshop, there was always adequate time



available for discussions, Questions & Answers and learner engagement (Votes' results : 65% yes always /almost always, 30% yes sometimes, 2,5% only rarely, 2,5% undecided).

For 82,5% of participants, the presented information was well balanced and consistently supported by a valid scientific evidence base. The event fulfilled the educational goals and expected learning outcomes for more than 60% of people (Votes' results : 62,5% very much, 32,5% somewhat).

According to the voters, we could improve the workshop with group work excercises, more key documents as the educational tool with all the scales or also by leaving more time for questions at the end of each session.

2-3 participants' testimonials

Can you provide one example how this event will influence your future practice?

"I am working in extraction neuroimaging biomarkers for ALS, this program provided me a wide range of innovative ideas starting from thinking on better approach on stratifying disease phenotypes, the critical need for better continuous clinical outcome measures for the disease" **Mohammed KHAMAYSA (Sorbonne university, France).**

"I will try especially to implement more scores into daily practice, as our outpatients are often seen by different doctors each time and this will help in getting a better overview of the patients' development" **Nele Baier (Essen university, Germany).**

What was the best aspect of this event?

"It was a great honor for me to be able to attend such a conference as a young neurologist. The topic and expertise of the lecturers, as well as the opportunity for personal interaction, were the best aspects" **Peter Krkoška (University Hospital Brno, Czech republic).**

"The revision of the outcomes, the pdf with the scales" **Julita Medina Cantillo (Hospital San Joan de Deu. Barcelona)**

if applicable a main result of the workshop

A manuscript about the outcome measures used in NMDs and discussed during the workshop will be written by all the speakers. We also discussed a lot about the possibility to organize, in two years maybe, a workshop on digital biomarkers in this field.

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

During the workshop, we discussed about the outcome measures used in a wide range of NMDs as myopathies, neuropathies, spinal muscular atrophy (SMA) and myasthenia gravis. The heterogeneity and rarity of these disorders are challenges for clinician to evaluate patients in clinical trials and therefore to develop effective diagnosis and treatment strategies.

One of the problems encountered in clinical trials is the lack of reliable and accurate measurements of clinical outcomes. The use of digital biomarkers could help us to solve much of this problem by providing invaluable tools to establish a more accurate diagnosis as well as monitor the progression of NMDs and the effect of a treatment or a therapeutic technique. They are frequently used outside of the clinical environment using home-based connected products including wearable, implantable, and ingestible devices and sensors. They offer the possibility to overcome many of the limitations of the traditional outcome measures and offer unique advantages like scale, capture frequency, data volume, measurement objectivity..

Thus, as we mentioned above, it is imperative to integrate these new measurements into our clinical trials and that is why we would like to discuss them at a future workshop that will be entirely dedicated to digital biomarkers.

To conclude, people who attended the event were very satisfied with the content and format of the workshop that we proposed. It was a beautiful opportunity for all the speakers present to share their knowledge and experiences about clinical outcome measures in NMDs but also to discuss about the future for clinical trials. As we can read through the testimonials of participants, this training will have a positive impact on their way of approaching clinical trials in the daily practice.

We encourage new neurologists, physiotherapists or researchers to join us during these workshops in order to improve clinical trials in this field.