

EJP RD Advisory Committee for Therapeutics (ACT) workshop (ERN-focused event)

Day 1

(times in CEST)

14:00 - Welcome (Victoria Hedley, Newcastle University)

14:15 - Introductions

14:30 - Introduction to the ACT model and to how it has benefited the neuromuscular community.

Speakers: Annamaria De Luca (TACT Chair, University of Bari), Volker Straub (TACT secretariat, Newcastle University), Cathy Turner (TACT secretariat, Newcastle University) and members of the TACT core committee (TBC)

15:15 - Patient organization perspective: added value of TACT 15:30 - An overview of the EJP RD vision to roll out ACTs to other rare disease communities. A presentation of the ACT toolkit will be provided.

Speaker: Joanne Lee (Newcastle University)

15:45 - Break

16:10 – 18:00

A practical demonstration of an ACT will take place. In advance of the workshop, participants will be given a mock application from a fictitious applicant seeking expert advice. The mock application will then be reviewed by members of the TACT committee during the workshop, enabling participants to understand how experts involved in different areas of drug development work together to provide comprehensive advice and recommendations to researchers involved in drug development programs.

Speakers: Annamaria De Luca (TACT Chair), Volker Straub (TACT secretariat) and members of the TACT committee (TBC)

Day 2

09:30 - Summary of Day 1 Presentations and priorities identified by participants' survey

10:00 - Case studies: How ERNs are optimizing clinical trials in their therapeutic area (2 case studies)

10:20 - Group Work: Participants will engage in group work to explore the potential of launching an ACT within their community (orientated to ERN thematic groupings). Participants will be asked to explore the opportunities, added value and any potential barriers they could foresee in launching an ACT.

12:00 - Break

12:30 - Plenary – reporting from Group Work and Discussion

Moderator: Victoria Hedley

Participants will seek to identify:

1. disease areas to prioritise
2. potential for cross-collaboration between ACTs, where experts' skills are transversal
3. seed funding opportunities to pilot an ACT within the EJP RD funding period
4. Related initiatives ACT to engage with

14:00 - Comfort break

14:15 - Funding Opportunities (part presentation, part reaction to group discussions)

14:40 – 15:00 - Final Conclusions and Evaluation of the Workshop