# Translational Summer School At A Glance

| Monday 2nd July 2018 | Tuesday 3rd July 2018 | Wednesday 4th July 2018 | Thursday 5th July 2018 | Friday 6th July 2018 |
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| | **SESSION 2: CLINICAL TRIALS**<br>**08:30 - 9:30 Introduction to clinical trials**<br>*(Michela Guglieri)*<br>**Objective:** introduce how and why clinical trials are conducted, the objective of different trial phases, trial design, primary endpoints, secondary endpoints, clinical significance, ethical concerns and informed consent | **09:00 - 10:00 Biomarkers**<br>*(Pietro Spitali & Andreas Roos)*<br>**Objective:** to explain why types of biomarkers exist and how they can be used in trial planning and as outcome measures, the regulatory perspective on biomarkers, highlighting ongoing networking efforts (ENMC workshops) | **SESSION 3:PATIENT COMMUNICATION WORKSHOP**<br>**09:00 - 10:00 Importance of patient communication**<br>*(Annemieke Aartsma-Rus)* | |
| | **09:30 - 10:30 Outcome Measures**<br>*(Anna Mayhew)*<br>**Objective:** overview of the different types of functional outcome measures used in clinical trials for neuromuscular, their opportunities and limitations, need for standardization and training, TREAT-NMD efforts in their area, natural history studies and their usefulness in trial design | **10:00 - 11:00 Showcase: validation of MRI as a biomarker in clinical trials**<br>*(Jordi Díaz Manera)*<br>**Objective:** introduce MRI as an outcome measure in trials, ongoing efforts to validate this biomarker for assessment of muscle quality in neuromuscular diseases | **10:00 - 11:00 Communication workshop by behavioral scientists**<br>*(Mats Wiert Postema & Stan Veldkamp)*<br>**Objective:** During this workshop participants will learn about unconscious processes, association and framing, which influence how what is communicated is received by the recipient. | |
| | **10:30 - 11:00 Tea/Coffee Break** | **11:00 - 11:30 Tea/Coffee Break** | **11:00 - 11:30 Tea/Coffee Break** | |
| **11:00 - 11.30 Arrival and Registration**<br>Tea/Coffee | **11:00 - 11:45 Showcase on outcome measure development**<br>*(Anna Mayhew)*<br>**Objective:** to outline the steps and stakeholders involved in developing and validating functional outcome measures that assess what patient find important using upper limb function outcome measure development for DMD and SMA as showcases | **11:30 - 12:15 Showcase: PROM development**<br>*(Nathalie Goemans)*<br>**Objective**: explain what happens after a drug is approved, marketing, postmarketing studies, reimbursement and challenges around drug access | ***11:30 - 12:30***<br>***Communication workshop by behavioral scientists cont.*** | **10:30 - 12:00 Participant presentations**<br>*(Teresinha Evangelista & Silvere van der Maarel)*<br>We ask each group of 3-4 participants to prepare a 10-15 minute talk<br>- Who they are and what they expected from the summer school<br>- The things they learnt - How this will influence their daily work<br>- What we should keep in future summer schools<br>- What we should drop/improve - What they were missing |
| **11:30 - 12:15 Welcome**<br>*(Teresinha Evangelista & Annemieke Aartsma-Rus)*<br>Brief round of introduction of all participants<br>**Objective:** to get to know one another | **11:45 -12:15 Feedback from TACT review session**<br>*(Annemieke Aartsma-Rus)*<br>**Objective:** align on strengths and weaknesses and outstanding questions identified by the groups | | | **12:00 - 12:30 Feedback and general discussion**<br>(Annemieke Aartsma-Rus &Teresinha Evangelista) |
| **12:15 - 13:00 Lunch** | **12:15 - 13:15 Lunch** | **12:15 - 13:15 Lunch** | **12:30 - 13:30 Lunch** | **12:30 - 13:30 End of summer school**<br>**Lunch & Departure** |
| **SESSION 1: TRANSLATIONAL LIFECYCLE**<br>**13:00 - 14:00 Overview of bench to bedside research**<br>*(Annemieke Aartsma-Rus)*<br>**Objective:** to outline the different steps of therapy development from idea, to proof of concept studies in model systems, to preclinical optimization studies, clinical trials, drug approval and post marketing surveillance studies | **13:15 - 14:45 Ethical discussion and Role Play**<br>*(Silvere van der Maarel, Becca Leary & Teresinha Evangelista)*<br>Participants will be provided with a scenario for a clinical trial plan for a drug to be tested in children. Different roles will be given to different participants.<br>**Objective:** gain insight in ethical discussions related to clinical trials and the perspectives of different stakeholders | **13:15 - 14:15 After EMA approval: lessons learned, challenges**<br>*(Ria Broekgaarden)*<br>Interactive workshop with Ria Broekgaarden (VSN Netherlands, PAB member EURO-NMD) to discuss challenges around drug access, start stop critera and drug migration | ***13:30 - 15:00***<br>***Communication workshop by behavioral scientists cont.*** | |
| **14:00 - 15:00 Tools of the trade for preclinical research**<br>*(Volker Straub & Annemieke Aartsma-Rus)*<br>**Objective:** outline different models in preclinical research, their opportunities and limitations, and the need for standardized tests and the TREAT-NMD advisory committee for therapeutics (TACT) | **14:45 - 15:15 Showcase on outcome measure development, labelling & marketing**<br>*(Annemieke Aartsma-Rus)*<br>**Objective:** to illustrate the consequences of using outcome measures limited to certain disease stages with regards to extrapolation and limited indications | **14:15 - 15:15 Role of patient organisations in research: examples, achievements from start up to registries**<br>*(Ria Broekgaarden)*<br>**Objective:** to learn how patients are equal partners in drug development and can be instrumental in the processes involved in drug development | | |
| **15:00 - 15:30 Tea/Coffee Break** | **15:15 - 15:45 Tea/Coffee Break** | **15:15 - 15:30 Tea/Coffee Break** | **15:00 - 15:30 Tea/Coffee Break** | |
| **15:30 - 16:30 How the regulatory system works**<br>*(Violeta Stoyanova-Benninska)*<br>**Objective:** explain the centralized system, how it is organized and how regulators decide whether drugs are eligible for marketing authorization, outline patient involvement in committees – focus on rare diseases | **15:45 - 16:45 TREAT-NMD tools to facilitate clinical trials**<br>*(Michela Guglieri & Becca Leary)*<br>**Objective:** to gain insight in available tools and services for planning and conducting clinical trials | **15:30 - 17:30 Clinical trial practicality forum**<br>*(Teresinha Evangelista)*<br>**Participants:** trial nurse, clinical trials clinician, clinical trial patients and patient representatives<br>In this panel session participants will briefly introduce themselves and their personal experience with being involved in a clinical trial as a health care professional or a patient.<br>- What is it like?<br>- What is challenging?<br>- What is burdensome?<br>- Where things going as expected?<br><br>This is followed by an interactive panel discussion where participants can ask questions and contribute to the discussion | | |
| **16:30 - 18:30 Mock TACT review session**<br>*(Annemieke Aartsma-Rus, Volker Straub, & Cathy Turner)*<br>**Objective**: learning to have a critical look at preclinical research. In this mock session participants will have been provided with a fictitious TACT application from a company planning a clinical trial in the NMD space. Participants will be split into groups of 8-10 participants to discuss the strengths and limitations and outstanding questions of the application. | | | **19:00 - LATE**<br>**Networking Dinner**<br>**Sabatinis' Newcastle Quayside** | |