

Translational Summer School Draft Agenda

	Monday 2nd July 2018	Tuesday 3rd July 2018	Wednesday 4th July 2018	Thursday 5th July 2018	Friday 6th July 2018	
MORNING	09:00	Feedback from TACT review session (Annemieke Aartsma-Rus) Objective: align on strengths and weaknesses and outstanding questions identified by the groups	Biomarkers (Pietro Spitali & Andreas Roos) 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)	PATIENT COMMUNICATION WORKSHOP Importance of patient communication (Annemieke Aartsma-Rus)		
	09:15					
	09:30					
	09:45	SESSION 2: CLINICAL TRIALS Introduction to clinical trials (Michela Guglieri) 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	Showcase: validation of MRI as a biomarker in clinical trials (Volker Straub) Objective: introduce MRI as an outcome measure in trials, ongoing efforts to validate this biomarker for assessment of muscle quality in neuromuscular diseases	Communication workshop by behavioral scientists (confirmed) Objective: During this workshop participants will learn about unconscious processes, association and framing, which influence how what is communicated is received by the recipient.		
	10:00	Tea/Coffee Break 30 mins	Tea/Coffee Break 30 mins	Tea/Coffee Break 30 mins		
	10:15	Arrival and registration Tea/Coffee	Ethical discussion and Role Play (Silvere van der Maarel, Becca Leary & Teresinha Evangelista) 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. Objective: gain insight in ethical discussions related to clinical trials and the perspectives of different stakeholders	Showcase: PROM development (Nathalie Goemans) Objective: explain what happens after a drug is approved, marketing, postmarketing studies, reimbursement and challenges around drug access		Communication workshop by behavioral scientists cont.
	11:00	Welcome (Teresinha and Annemieke) Brief round of introduction of all participants Objective: to get to know one another	Lunch 45 Mins	Lunch 60 Mins		Communication workshop by behavioral scientists cont.
	11:15	Feedback and general discussion (Annemieke Aartsma-Rus and Teresinha Evangelista)				
	11:30	Lunch 45 Mins	Lunch 60 Mins	Lunch 60 Mins		End of summer school Lunch & Departure
	11:45	SESSION 1: TRANSLATIONAL LIFECYCLE Overview of bench to bedside research (Annemieke Aartsma-Rus) 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				
12:00	Tools of the trade for preclinical research (Volker Straub & Annemieke Aartsma-Rus) 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)	Outcome Measures (Anna Mayhew) 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	Role of patient organisations in research: examples, achievements from start up to registries (Ria Broekgaarden) Objective: to learn how patients are equal partners in drug development and can be instrumental in the processes involved in drug development	Communication workshop by behavioral scientists cont.		
12:15	Tea/Coffee Break 30 mins	Showcase on outcome measure development and labelling and marketing (Annemieke Aartsma-Rus) Objective: to illustrate the consequences of using outcome measures limited to certain disease stages with regards to extrapolation and limited indications	Tea/Coffee Break 15 mins	Tea/Coffee End of Day		
12:30	How the regulatory system works (Violeta Stoyanova-Benninska) 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	Showcase on outcome measure development (Anna Mayhew) 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	Clinical trial practicality forum (Teresinha Evangelista) Participants: trial nurse, clinician involved in clinical trials, patients involved in clinical trial, patient representatives 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. What is it like? What is challenging? What is burdensome? Where things going as expected? This is followed by an interactive panel discussion where participants can ask questions and contribute to the discussion			
12:45	Mock TACT review session (Annemieke Aartsma-Rus, Volker Straub, & Cathy Turner) 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.	TREAT-NMD tools to facilitate clinical trials (Michela Guglieri & Becca Leary) Objective: to gain insight in available tools and services for planning and conducting clinical trials				
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