Translational Summer School 2019

Final Program

Monday 1st July

Time	Activity
11:00-11:30	Arrival and registration
11:30-11:45	Welcome (***Teresinha, Silvere, Andoni and Annemieke***) *This will include a brief outline of the objectives of the summer school and the week schedule*
11:45-12:15	Brief round of introduction
12:15-13:00	Lunch
	Session 1 : Introduction
13:00-14:00	Overview of current state of the art of NMD therapies and management (***Andoni Urtizberea***) *Objective: give participants a global overview of the different groups of NMDs (genetic and acquired) and the current care and management practices (45 minute talk, 15 minute discussion)*
14:00-14:20	Brief 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 (15 minute talk, 5 minute discussion)*
	Challenges for rare disease therapy development and networking solutions (***Teresinha Evangelista***) *Objective: introduce the challenges of rare disease therapy development and how TREAT-NMD and EURO-NMD networks address this (45 minute talk, 15 minute discussion)*
15:20-15:45	Tea/coffee break

	Session 2 : Preclinical Research
15:45-16:45	Tools of the trade for preclinical research (***Volker Straub and Annemieke Aartsma-Rus***) *Objective: outline different models used in preclinical research, their opportunities and limitations, and the need for standardized tests and the TREAT-NMD advisory committee for therapeutics (TACT)) (2 20 minute talks, 20 minutes discussion)*
16:45-17:00	Introduction to TACT mock up session (***Annemieke Aartsma-Rus***)
17:00	End of Day

Tuesday 2nd July

Time	**Activity**
09:00-10:00	Self-study for TACT mock review session
10:00-11:00	When to move to a clinical trial? TACT mock review session (***moderated by Annemieke Aartsma-Rus and Volker Straub***) *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*
11:00-11:30	Coffee
	Session 3: clinical research
11:30-12:30	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; 45 minute talk, 15 minutes discussion*
12:30-13:30	Lunch
13:30-14:30	Ethical discussion role play (***moderated by Annemieke Aartsma-Rus and Teresinha Evangelista***)

	Participant 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.* *Reading of provided material ~5 minutes;* *Discussion ~40 minutes,* *Evaluation (leaving roles behind) ~15 minutes*
14:30-15:30	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, 45 minute talk, 15 min discussion time*
15:30-16:00	Coffee break
16:00-16:40	Industry perspective on drug development for rare diseases (***Thomas de Vlaam***) *Objective: provide the perspective of pharmaceutical companies on drug development for rare diseases, which challenges do they face, how do they deal with them; 30 minute talk, 10 minutes discussion time*
16:40-17:40	Clinical trial practicality forum (***Teresinha Evangelista and Tiago Gomes***) *Objective: provide insight in the logistics of running a clinical trial (recruitment, treating, dealing with patient expectations, informed consent etc)*
17:40	End of the day

Faculty Dinner

Wednesday 3rd July

Time	**Activity**
	Session 4: Outcome measures
09:00-10:00	Outcome measures (***Anna Mayhew***) *Objective: outline of requirements of outcome measures used in clinical trials, how to select the best outcome measure for a pivotal trial; using real life examples of successful and failed trials (e.g. drisapersen 6MWT) 45 minute talk, 15 minute discussion)*
10:00-10:45	Showcase on outcome measure development (reachable workspace) (***Anna Mayhew***) *Objective: to outline the steps and stakeholders involved in developing and validating a functional outcome measure (select one – indicate the same applies for all); 30 minute talk, 15 minute discussion*
10:45-11:00	Coffee break
11:00-12:00	Showcase: validation of MRI as a biomarker in clinical trials (***Hermien Kan***) *Objective: introduce MRI as an outcome measure in trials, ongoing efforts to validate this biomarker for assessment of muscle quality in neuromuscular diseases, 45 minute talk, 15 minute discussion*
12:00-13:00	TREAT-NMD tools to facilitate clinical trials (***Michela Guglieri and Becca Leary***) *Objective: to gain insight in available tools and services for planning and conducting clinical trials* *Michela Guglieri ~ 20 minutes (standards of care, networking, interaction with regulators, clinical trial capacity hub model)* *Becca Leary ~25 minutes (patient registries and CTSR)* *Discussion ~15 minutes*
13:00-14:00	Lunch
14:00-14:30	Showcase on outcome measure development and labeling 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 (20 minute talk, 10 minutes discussion)
14:30-15:30	Biomarkers (***Pietro Spitali***) *Objective: explain the different types of biomarkers, how they can be used in trial planning and as outcome measures, the regulatory perspective on biomarkers, highlighting ongoing networking efforts (45 minute talk, 15 minute discussion)*
15:30-16:00	Coffee Break
16:00-17:00	Showcase: PROM development (***Nathalie Goemans***) *Objective: explain what is involved in developing a patient reported outcome measure using the DMD PROM development as a showcase, 35 minute talk, 10 minute discussion*
	Session 5 : Post marketing
17:00-18:00	After approval: Proms and HTA (***Ria Broekgaarden***) *Objective: explain what happens after a drug is approved, marketing, postmarketing studies, reimbursement and challenges around drug access, 45 minute talk, 15 minutes discussion*
18:00	End of day

Thursday 4th July

Time	Activity
	Session 6 Patient engagement
09:00-10:00	How patients can help your research from bench to bedside (***Ria Broekgaarden***) *Objective: Examples are given of how patients can be involved in helping with all steps of therapy development, to underline that patients or not only study objects, but also active participants*
10:00-11:00	Translating science to the non initiated (***Maarten Keulemans***) *Objective: Participants will gain insight in how to clearly explain science to people without a scientific background – with a focus on patients where the added challenge is not to overpromise or raise false expectations; 45 minute lecture, 15 minute discussion*
11:00-11:30	Coffee break
11:30-12:30	Translating science to inform patients *Objective: participants are asked to bring a scientific publication on a potential therapy of their choice. They will now have to summarize this into a 500-word blog understandable by patients and their families*
12:30-13:30	Lunch
13:30-15:30	Presenting science to patients *Objective: participants are split in groups of 4 and will be provided with a scientific paper. It is their task to generate a presentation (~10-15 minutes) to inform patients of the scientific findings in a clear and objective manner*
15:30-16:30	Presentations from each of the groups (***facilitated by Silvere van der Maarel***) *Objective: each of the groups presents their presentation, the other participants are the audience and listen as if they were a patient*
16:30	Feedback and discussion (**facilitated by Silvere van der Maarel**)
	Network Dinner

Friday 5th July

Time	Activity
09:00-10:30	Participants work on preparing their final presentation
10:30-12:00	Participant presentations (***Chaired by Teresinha Evangelista and Silvere van der Maarel***) We ask groups of 2 participants to prepare a 10-15 minute talk - Who they are and what they expected from the summer school - The things they learnt - How this will influence their daily work - What we should keep in future summer schools - What we should drop/improve - What they were missing
12:00-12:30	Feedback on blogs, award for best blog and general discussion (***Chaired by Annemieke Aartsma-Rus and Teresinha Evangelista***)
12:30	End of Summer School
	Lunch and Departure