

**** Preliminary programme ****

Friday 15 February 2019

12:30–14:00	Lunch
14:00-14:45	Section I: <u>Introduction</u>
14:00-14:15	Alexandra Breukel; Introduction to ENMC meeting
14:15-14:30	Kenneth Fischbeck; Overview on SBMA
14:30-14:45	Maria Pennuto; Aims of workshop
14:45-16:00	Section II: <u>Disease mechanisms: From AR structure to function in SBMA</u> Chairs: Angelo Poletti & Xavier Salvatella
14:45-15:00	Xavier Salvatella; How polyQ tract length modulates the structural and oligomerization properties of the androgen receptor
15:00-15:15	Angelo Poletti; The routing system of the protein quality control in the clearance of mutant androgen receptor
15:15-15:30	Maria Polanco; Role of cell cycle molecules in the pathogenesis of SBMA
15:30-16:00	Round table
16:00-16:30	Break
16:30-18:30	Section III: <u>Peripheral polyglutamine-expanded AR toxicity</u> Chairs: Carlo Rinaldi & Linda Greensmith
16:30-16:45	Carlo Rinaldi; Extra motor-neuron involvement in SBMA: expanding the phenotype/refining the target
16:45-17:00	Maria Pennuto Metabolic alterations in SBMA mice
17:00-17:15	Bilal Malik; Title tbe
17:15-17:30	Linda Greensmith; Uncovering pathomechanisms of muscle atrophy from a mouse model of spinal and bulbar muscular atrophy
17:30-18:30	Round table

Saturday 16 February 2019

09:00-11:00	Section IV: <u>Preclinical approaches to reduce polyglutamine-expanded AR toxicity</u> Chairs: Aria Baniahmad and Manuela Basso
09:00-09:15	Aria Baniahmad; Structures and Characteristics of novel Androgen Receptor Antagonists
09:15-09:30	Giuseppe Ronzitti; Development of AAV gene therapy approaches from the bench to the bedside
09:30-09:45	Manuela Basso; Targeting PRMT6 to attenuate spinal and bulbar muscular atrophy: a silencing approach
09:45-10:00	Andrea Caricasole; Interrogating post-translational modifications as opportunities for therapeutic approaches in polyglutamine diseases
10:00-10:15	Paola Rusmini; Title tbe
10:15-11:00	Round table
11:00-11:30	Break
11:30-12:30	Section V: <u>Towards a EU registry for SBMA</u> Chair: Dr Carlo Rinaldi
11:30-11:45	Davide Pareyson; Towards a European registry for SBMA
11:45-12:30	Round table
12:30-14:00	Lunch
14:00-16:00	Section VI: <u>Clinical development for SBMA</u> Chairs: Dr John Vissing and Dr Gianni Sorarù
14:00-14:15	Kenneth Fischbeck; Therapeutics development for SBMA
14:15-14:30	John Vissing; Natural history and definition of appropriate outcome measures for SBMA in upcoming clinical trials
14:30-14:45	Gianni Sorarù; A randomized clinical trial with clenbuterol in SBMA
14:45-15:00	Illana Gozes; Nerve cell protective peptide drug design and development
15:00-16:00	Round Table
16:00-16:30	Break
16:30-17:30	Section VII: <u>Biomarkers for SBMA</u> Chair: Dr John Vissing
16:30-16:45	Francois Pradat; Spinal cord imaging: a potential biomarker in SBMA?
16:45-17:00	Pietro Fratta; An update on blood biomarkers and muscle imaging in SBMA
17:00-17:30	Round table
17:30-18:30	Section VI: <u>Patients' perspective</u> Chair: Luca Zampedri
	Marco Bertolotti & Gianni Fabris: Towards a KDA in Italy
	Edward Meyertholen, patients perspective
	Luca Zampedri, patients perspective

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Sunday 17 February 2019

09:00-10:30	Selected chairs of each section will summarize the topics of discussion and will drive discussion of the future steps.
10:30-12:00	Lay summary of meeting and report for the journal Neuromuscular Disorders
12:00	Closing remarks, lunch and departure