

#### Programme committee:

Dr. J. Andoni URTIZBEREA (Institute of Myology, Paris, France)
Nur Rocio VILAR QUILES (Neuro- Myology Service, Pitié-Salpêtrière Hospital, Paris)
Dr. Gorka FERNANDEZ-EULATE (Neuro- Myology Service, Pitié-Salpêtrière Hospital, Paris)
Harmen REYNGOUDT (CEEN, Institute of Myology, Groupe Hospitalier Pitié-Salpêtrière, Paris)
Dr Andreea SEFERIAN (I-Motion, Institute of Myology, Trousseau Hospital, Paris)
Dr Stéphane VASSILIPOULOS (CDR, Institute of Myology, Pitié-Salpêtrière Hospital, Paris)
Jorge BEVILACQUA (Neurology Department, University Hospital of Chile, Santiago)

Target audience (25 participants max.)

- Healthcare providers (HCP's)
- Physicians & allied professionals
- Researchers/Scientists
- Industry employees

#### Aim:

To update participants on the current trends and innovations in myology but also equip them with the practical tools necessary for effective diagnosis and treatment of neuromuscular disorders, ultimately enhancing patient care and fostering ongoing professional development.



# MONDAY 16th JUNE (DAY 1)

08.45- 09.00	Introduction and welcome (J. Andoni Urtizberea, Nur Villar-Quiles, Jorge Bevilacqua) Objective: Introduction of participants and organizers; layout of the program and lograping objectives
09.00- 9.45	program and learning objectives Introduction to clinical myology (Jorge Bevilacqua)
	<b>Objective:</b> To provide participants with a foundational understanding of
	clinical myology, focusing on the diagnosis, management, and treatment of
	muscle-related disorders, and the clinical relevance of muscle pathology in
	neuromuscular diseases. (35'talk, 15' discussion)
9.45-10.30	Update on muscular dystrophies - including LGMD (Gorka Fernandez)
	<b>Objective:</b> To provide participants with a comprehensive understanding of
	the current classifications, genetics, and therapeutic options for muscular
	dystrophies, with a focus on LGMD. (35'talk, 15' discussion)
10.30-10.45	Coffee Break
	Case studies (session 1)
10.45- 12.45	(J. Andoni Urtizberea, Gorka Fernández, Nur Villar Quiles, Jorge Bevilacqua)
12.45- 14.00	Lunch Break (room 1)
14.00- 16.15	Hands-on workshops and on-site visits (Day 1)
16.15- 16.30	Coffee Break
	Elucidating rare neurodegenerative/neuromuscular diseases by using
16.30- 17.15	new technologies (Andrea Cortese)
	<b>Objective:</b> To explore how new technologies like genomics and
	bioinformatics are advancing the diagnosis and understanding of rare neurodegenerative and neuromuscular diseases (35'talk, 15' discussion)
	End of day 1- Q&A with drinks and small bites; meet the speakers



## **TUESDAY 17th JUNE (DAY 2)**

09.00- 9.45	Charcot-Marie-Tooth disease (Tanya Stojkovic)
	<b>Objective:</b> To update participants on the latest developments in the
	pathophysiology, diagnosis, and treatment strategies for Charcot Marie
	Tooth disease. (35'talk, 15' discussion)
9.45-10.30	MRI Fingerprinting for fast quantitative MRI of the skeletal muscles
	(Constantin Slioussarenko)
	<b>Objective:</b> To familiarize participants with the technique of MRI
	fingerprinting and its application in the rapid, quantitative analysis of
	skeletal muscle conditions. (35'talk, 15' discussion)
10.30-10.45	Coffee Break
10.45- 12.45	Case studies (session 2) (J. Andoni Urtizberea, Gorka Fernández, Nur Rocio Villar Quiles, Jorge Bevilacqua)
12.45- 14.00	Lunch Break (room 1)
14.00- 16.15	Hands-on workshops and on-site visits (Day 2)
16.15- 16.30	Coffee Break
16.30- 17.15	Outcome measures (Simone Birnbaum) Objective: To teach participants how to select and apply appropriate outcome measures to assess progression and treatment efficacy in neuromuscular diseases. (35'talk, 15' discussion)
	End of day 2- Q&A with drinks; meet the speakers



## WEDNESDAY 18th JUNE (DAY 3)

09.00- 9.45	Myasthenia Gravis and novel therapies (Anthony Béhin)
	<b>Objective:</b> To provide an overview of Myasthenia Gravis and present novel
	treatment options that are emerging in clinical practice. (35'talk, 15'
	discussion)
9.45-10.30	Therapeutics in SMA: an update (Andreea Seferian)
	<b>Objective:</b> To explore the latest therapeutic advancements in Spinal
	Muscular Atrophy, including gene therapies and disease-modifying
	treatments. (35'talk, 15' discussion)
10.30-10.45	Coffee Break
	Case studies (session 3)
10.45- 12.45	(J. Andoni Urtizberea, Gorka Fernández, Nur Rocio Villar Quiles, Jorge
	Bevilacqua)
12.45- 14.00	Lunch Break (room 1)
14.00- 16.15	N/A (spare time)
16.15- 16.30	N/A (spare time)
16.30- 17.15	N/A (spare time)
	End of day 3



# THURSDAY 19th JUNE (DAY 4)

09.00- 9.45	Update in metabolic myopathies (Edoardo Malfatti)
	<b>Objective:</b> To discuss recent advances in the identification, diagnosis, and
	management of metabolic myopathies, including enzyme replacement
	therapies. (35'talk, 15' discussion)
9.45-10.30	Clinical Trials (Michella Ibrahim)
	<b>Objective:</b> To guide participants in understanding the key aspects of
	designing, conducting, and interpreting clinical trials in neuromuscular
	research. (35'talk, 15' discussion)
10.30-10.45	Coffee Break
	Case studies (session 4)
10.45- 12.45	(J. Andoni Urtizberea, Gorka Fernández, Nur Villar Quiles, Jorge Bevilacqua)
12.45- 14.00	Lunch Break (Room 1)
14.00- 16.15	Hands-on workshops and on-site visits (Day 4)
16.15- 16.30	Coffee Break
	Myotonic dystrophies and therapies (Denis Furling)
16.30- 17.15	<b>Objective:</b> To update participants on the pathophysiology and available
	therapies for myotonic dystrophies, including current clinical trial data.
	(35'talk, 15' discussion)
19.00-22.00	Networking Dinner



# FRIDAY 20th JUNE (DAY 5)

09.00- 9.45	Selenopathies and other Congenital muscular dystrophies (Ana Ferreiro)
	<b>Objective:</b> To educate participants on the clinical features, molecular basis,
	and treatment approaches for selenopathies and other congenital muscular
	dystrophies. (35'talk, 15' discussion)
9.45-10.30	Robotics (Romain Feigean)
	<b>Objective:</b> To explore the role of robotic technologies in the rehabilitation
	and treatment of neuromuscular diseases, focusing on enhancing patient
	mobility and function. (35'talk, 15' discussion)
10.30-10.45	Coffee Break
	Case studies (session 5)
10.45- 12.45	(J. Andoni Urtizberea, Gorka Fernández, Nur Villar Quiles, Jorge Bevilacqua)
12.45- 14.00	Lunch Break (room 1)
14.00- 16.15	Hands-on workshops and on-site visits (Day 5)
16.15- 16.30	Coffee Break (+preparation participant presentations)
	Quizz, Evaluation, Participant presentations (chaired by Andoni Urtizberea)
	We ask groups of 5 participants to prepare a talk
	<ul> <li>Who they are and what they expected from the summer school</li> </ul>
16.30- 17.30	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
	Feedback and general discussion. Farewell and departure.



#### HANDS-ON WORKSHOPS AND ON-SITE VISITS

#### 1/ SALPETRIERE HOSPITAL

- 1.1 Muscle Histology Morphological Unit (Teresinha Evangelista)
- 1.2 Myogenetics- Bioinformatics (France Leturcq)

1.3 Electrophysiology (Sarah Leonard-Lewis, Tanya Stojkovic, Marion Hezode (EMG salle Service de Neuro-Myologie)- canalopathy ,+test etc)

- 1.4 Muscle Electron Microscopy (Stéphane Vassilopoulos)
- 1.5 Historical tour of the Salpêtrière Hospital (J.Andoni Urtizberea)
- 1.6 Neuromuscular Physiology and Evaluation Laboratory (Jean Yves Hogrel)
- 1.7 Respiratory management (Hélène Prigent)

1.8 Muscle Imaging (Harmen Reyngoudt) (Laboratoire d'Imagerie et de Spectroscopie par résonance magnétique nucléaire, Babinski building)

#### 2/I-Motion

Clinical Trial Unit I-Motion Pediatric (Marina Colella, Andreea Seferian)