Expert Masterclass on Limb Girdle Muscular Dystrophy

Wednesday 23rd - Thursday 24th September 2020

Programme

Meeting Objectives

- Deliver high quality educational and interactive meeting for providers diagnosing or managing the care of patients with the limb girdle muscular dystrophies by providing information on the latest in diagnosis, natural history, and care discussions.
- Provide a platform for health care providers to share ideas, challenges and successes in delivering optimal care to people with LGMD.
- Provide an overview of the current clinical trial landscape and the emerging implications on care

Meeting Format

- Wednesday 23rd September & Thursday 24th September

Chairs

- **Professor Jordi Diaz Manera**
- **Dr Lindsay Alfano**

Please note more details will follow of how each session will be delivered

Wednesday 23rd September	
Talk	**Speaker**
Welcome, programme overview and introduction to TREAT-NMD	**Professor Jordi Diaz Manera,** Neurologist, John Walton Muscular Dystrophy Research Centre, UK **Dr. Lindsay Alfano,** Physical Therapist, Nationwide Children's Hospital
Patient perspective • Psychosocial impact	**TBC**
LGMD Overview, Definitions & Classification	**Dr. Matthew Wicklund**, Neurologist, University of Colorado, USA
Frequency and distribution of LGMDs across the world. • Insights from the NGS screening Focus in LATAM • Recently published papers in USA	**Dr. Tahseen Mozaffar**, Neurologist, UC Irvine, USA
Diagnostic journey of the LGMD patient. • Differential diagnosis – other neuromuscular disorders with limb girdle patterns of weakness (Pompe, adult-onset SMA, etc)	**Dr. Jeff Statland**, Neurologist, Kansas University, USA
Most frequent recessive forms of LGMD R1 CAPN3, R2 DYSF, R12 ANO5 • Including an introduction to Pathophysiology	**Dr. Nick Johnson**, Neurologist, Virginia Commonwealth University, USA
Most frequent recessive forms of LGMD R3-6SGCA-G, and R9 FKRP and dystroglycanopathies • Including an introduction to Pathophysiology	**Dr. Kathy Mathews**, Neurologist University of Iowa, USA
Dominant LGMD ** or R7, R10? • Including an introduction to Pathophysiology	**Dr. C. Chris Weihl**, Neurologist, University of Washington – St. Louis, USA
Uses and applications of the muscle biopsy and other molecular methods for diagnosis in LGMD	**Dr. Jim Dowling**, Neurologist, The Hospital for Sick Children, Canada
Uses and applications of muscle MRI in LGMDs	**Professor Jordi Diaz Manera,** Neurologist, John Walton Muscular Dystrophy Research Centre, UK
Best practice on genetic diagnostic approaches When to use NGS, WES or WGS?	**Dr. Monkol Lek**, Assistant Professor, Yale School of Medicine, USA
Clinical trials landscape & emerging therapies	**Dr. Kathryn Wagner**, Neurologist, Kennedy Krieger Institute, USA

Thursday 24th September	
Natural history of the most frequent LGMDs	**Dr. Lindsay Alfano**, Physical Therapist, Nationwide Children's Hospital **Dr. Meredith James**, Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK
Best practice for Care management 1. Orthopaedics care	**Dr. Linda Lowes,** Physical Therapist, Nationwide Children's Hospital
Best practice for Care management - Cardiological care	**Dr. Linda Cripe,** Cardiologist, Nationwide Children's Hospital, USA
Best practice for Care management - Respiratory care	**Dr Grace Paul,** Paediatric Pulmonologist, Nationwide Children's Hospital, USA
Registries • **Why are they needed** • **Practicalities of setting up a registry**	**Dr Victoria Hodgkinson,** National Program Manager, TREAT-NMD SMA Registries, Canada

Workshop 1. Physiotherapy and other clinical care considerations
 	
Dr. Lindsay Alfano, Physical Therapist, Nationwide Children's Hospital
 	
Dr. Linda Lowes, Physical Therapist, Nationwide Children's Hospital
 	
Dr. Meredith James, Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK
 	
Dr. Anna Mayhew, Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK
 	
• **Physiotherapy clinical care considerations**
  ○ Exercise, stretching, bracing, equipment
• **Outcome measures**
  ○ Strength and functional testing
  ○ Patient reported measures
• **Occupational therapy**
• **General nutrition**
  ○ Examining the scientific evidence of some common diets and supplements
	Workshop 2. Exemplary clinical cases
	
	Professor Jordi Diaz Manera, Neurologist, John Walton Muscular Dystrophy Research Centre, UK
	
	Dr. C. Chris Weihl, Neurologist, University of Washington – St. Louis, USA
	
	Dr. Nick Johnson, Neurologist, Virginia Commonwealth University, USA
	
	Dr. Jim Dowling, Neurologist, The Hospital for Sick Children, Canada
	
	• **Demonstration and group discussion 3-4 case studies**