

TREAT-NMD Limb Girdle Muscular Dystrophy Australasia Expert Masterclass

11th & 12th May 2022

Australia & Asia Virtual – *online*

Programme

Masterclass Main Goals

- Deliver an interactive teaching course of excellence aimed at health personnel involved in the diagnosis, management, and treatment of patients with limb girdle muscular dystrophies (LGMD), by providing updated information on the diagnosis, natural history, and treatment of the disease.
- Provide a platform to promote the exchange of ideas, experiences, and common challenges in delivering the highest standard of care to patients with LGMD.
- Provide an updated overview on the development of different therapeutic trials as well as state of the art emerging therapies for LGMD, and the implications of its implementation.

Masterclass Format

This event will be delivered in a completely virtual way using our interactive virtual platform, Whova. The main language of the Masterclass will be in English. Interpreters will be available to provide translations to delegates across other languages inclusive of Chinese, Japanese & Korean. There will be a series of lectures given by experts, two interactive workshops and group discussions between experts and participants, through interactive question and answer sessions.

Day 1

The first day of the course will begin with the testimony of patients and parents of patients affected by LGMD, who will discuss the impact of Limb Girdle muscular dystrophies on their lives. This will be followed by a series of lectures on the different aspects of LGMDs given by experts. Attendees will have the opportunity to ask questions and interact with patients and speakers.

Day 2

On the second day of the course there will be additional lectures on specific aspects of the management of LGMD and an update on state of the art of care practices. At the end of each lecture there will be time dedicated to questions from the audience. Next, the attendees will be separated into two groups to participate in one of the two interactive workshops that will take place simultaneously.

Chairs

- **Professor Ichizo Nishino**, National Centre of Neurology and Psychiatry, Japan
- **Dr Gina Ravenscroft**, University of Western Australia, Australia

Pre-recorded Sessions

These pre-recorded webinars will be made available to delegates in advance of the live masterclass.

Session Duration	Session Title and subject	Speaker suggestions
30 min	LGMD Overview, Definitions & Classification.	Khean Jin Goh, University of Malay, Malaysia
30 min	Frequency and distribution of LGMDs across the world.	Richard Roxburgh, University of Auckland, New Zealand
40 min	Clinical trials landscape & emerging therapies.	Yuko Motohashi, National Centre for Neurology & Psychiatry, Japan
25 min	Natural History of Dysferlinopathy.	Jin-Hong Shin, Pusan National University Yangsan Hospital, Korea
25 min	Natural History of Calpainopathy.	Chongbo Zhao, Huashan Hospital, Fudan University, China
25 min	Natural History of Sarcoglycanopathies.	Wen-Chen Liang, Kaohsiung Medical University, Taiwan
30 min	Standard of Care: Enabling the LGMD patient.	Umapathi Thirugnanam, National Neuroscience Institute, Singapore
25 min	Registries. Why are they needed and the practicalities of setting up a registry?	Oranee Sanmaneechai, Siriraj Hospital Mahidol University, Thailand
30 min	Pompe Disease: Overview, Epidemiology and pathogenesis.	Nadine van der Beek, Erasmus MC University Medical Centre, Netherlands
30 min	Pompe Disease: Clinical Trials landscape & new emerging therapies.	Mark Roberts, Greater Manchester Neurosciences Unit, UK

Min	Time Zone: Sydney, AEST	Time Zone: Tokyo, JST	DAY 1 Wednesday 11 th May, 2022 (4 hours 50 mins running time)	(4 hours 50 mins running time)
5 min	16:00-16:05	15:00 - 15:05	Welcome, programme overview and introduction to TREAT-NMD	Ichizo Nishino & Gina Ravenscroft
20 min	16:05-16:25	15:05-15:25	Patient perspectives (16:05 – 16:15 AEST) <ul style="list-style-type: none"> Impact of LGMD on their lives and journey 	TBC
			Parent & Patient perspectives (16:15 – 16:25 AEST) <ul style="list-style-type: none"> Impact of LGMD on their lives and journey 	Daniel & Rick Ferguson, Daniel Ferguson Foundation, Australia
5 min	16:25-16:30	15:25 - 15:30	Q & A	
25 min	16:30 - 16:55	15:30 - 15:55	Diagnostic journey of the LGMD patient. <ul style="list-style-type: none"> Differential diagnosis – other neuromuscular disorders with limb girdle patterns of weakness, (adult-onset SMA, inflammatory myopathies etc.) 	Phillipa Lamont, Royal Perth Hospital, Australia
5 min	16:55 - 17:00	15:55 – 16:00	Q & A	
25 mins	17:00-17:25	16:00 – 16:25	Late onset Pompe Disease as a mimic of LGMD. <ul style="list-style-type: none"> Diagnostic process of Pompe Current treatment options 	Jordi Diaz-Manera, Newcastle University, UK
5 mins	17:25-17:30	16:25-16:30	Q & A	
25 min	17:30-17:55	16:30 – 16:55	Recessive forms I: DYSF, CAPN3, Sarcoglycanopathies <ul style="list-style-type: none"> Including an introduction to Pathophysiology 	Zhaoxia Wang, Peking University First Hospital, China
5 min	17:55 - 18:00	16:55 – 17:00	Q & A	
15 min	18:00-18:15	17:00 - 17:15	Break	Break

25 min	18:15-18:40	17:15 - 17:40	Recessive forms II: FKRP, dystroglycanopathies <ul style="list-style-type: none"> Including an introduction to Pathophysiology 	Hui Xiong, Peking University First Hospital, China
5 min	18:40-18:45	17:40 - 17:45	Q & A	
25 min	18:45-19:10	17:45 - 18:10	Less frequent forms of LGMD including titinopathy, LGMD-myasthenia, new genes.	Wenhua Zhu, Huashan Hospital, Fudan University, China
5 min	19:10 - 19:15	18:10 - 18:15	Q & A	
25 min	19:15-19:40	18:15-18:40	Muscular biopsy in the diagnosis of LGMD	Jantima Tanboon, Siriraj Hospital, Mahidol University, Thailand
5 min	19:40-19:45	18:40 - 18:45	Q & A	
25 min	19:45-20:10	18:45 - 19:10	MRI in the diagnosis of LGMD	Chuanzhu Yan, Qilu Hospital, Shandong University, China
5 min	20:10 - 20:15	19:10 - 19:15	Q & A	
25 min	20:15-20:40	19:15 - 19:40	The role of NGS in neuromuscular diseases	Mark Davis, Pathwest Laboratory Medicine, Australia
5 min	20:40-20:45	19:40 - 19:45	Q & A	
5 min	20:45-20:50	19:45 - 19:50	Summary	Chairs

Mins	Time Zone: Sydney, AEST	Time Zone: Tokyo, JST	Day 2- Thursday 12 th May 2022. 5 hours running time	
5 min	16:00 – 16:05	15:00 - 15:05	Welcome	Ichizo Nishino & Gina Ravenscroft
25 min	16:05 – 16:30	15:05 – 15:30	Best practice for care management: Orthopaedics	Kristy Rose, ATOM International Ltd, Australia
5 min	16:30 – 16:35	15:30 – 15:35	Q & A	

25 min	16:35 – 17:00	15:35 – 16:00	Best practice for care management: Respiratory	Yuka Ishikawa, National Hospital Organisation Hokkaido Medical Centre, Japan
5 min	17:00 – 17:05	16:00 – 16:05	Q & A	
25 min	17:05 – 17:30	16:05 – 16:30	Best practice for care management: Cardiological	Koichi Kimura, University of Tokyo, Japan
5 min	17:30 – 17:35	16:30 – 16:35	Q & A	
15 min	17:35 – 17:50	16:35 – 16:50	Break	Break
3 hrs	17:50 – 20:50	16:50 – 19:50	<p style="text-align: center;">Workshop 1 Physiotherapy and other Clinical Care Considerations</p> <p>Speakers: Kristy Rose, ATOM International Ltd, Australia Meredith James, Newcastle University, UK</p> <p>An interactive workshop which will include:</p> <p>Physiotherapy clinical care considerations</p> <ul style="list-style-type: none"> • Exercise, stretching, bracing and equipment <p>Outcome measures</p> <ul style="list-style-type: none"> • Strength and functional testing • Patient reported measures <p>Occupational therapy</p> <p>General nutrition</p> <ul style="list-style-type: none"> • Examining the scientific evidence of some common diets and supplements 	<p style="text-align: center;">Workshop 2 Exemplary Clinical Cases</p> <p>Speakers: Roula Ghaoui, Royal Adelaide Hospital, Australia Gina Ravenscroft, University of Western Australia Phillipa Lamont, Royal Perth Hospital, Australia</p> <p>Demonstration and group discussions around clinical cases. Group interaction, feedback, Q&A</p>
10 min	20:50 – 21:00	19:50 – 20:00	Meeting close	Chairs