

SUNDAY 9 JULY 2017

- 7.30 – 8.30** **Clinical Trial Updates** AUDITORIUM
CMTR Day 1
Chairs: **David R. Cornblath** and **Mary M. Reilly**
- 7.30 Safety And Efficacy of Inotersen in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy (hATTR-PN)
Teresa Coelho
- 7.45 The CMT Patient Experience and the Impact of Patient Reported Outcomes on Clinical Research and Trials
Allison Moore
- 7.55 The Effects of a Novel Exercise and Mobility Program on Clinical, Performance, and Patient Reported Outcome Measures in Patients with CMT
James Nussbaum
- 8.00 The Effects of a Gravity Supported Exercise Program on Performance, Clinical, and Patient Reported Outcome Measures in Patients with CMT
James Nussbaum
- 8.05 The German Charcot-Marie-Tooth Disease Network (CMT-NET): Disease Severity and Prognostic Biomarkers from Blood and Skin of CMT1a Patients
Thomas Prukop
- 8.10 Closing & remarks

Monday 10 July 2017

- 7.30 – 8.30** **Clinical Trial Updates** AUDITORIUM
CIDP
Chairs: **David R. Cornblath** and **Mary M. Reilly**
- 7.30 PATH study: Additional results
Orell Mielke
- 7.40 Rozanolixizumab: Development of a novel immunomodulator
Peter Kiessling
- 7.50 The DRIP study: A dose response trial of IVIg in CIDP
Krista Kuitwaard
- 7.55 Safety of IVIG in children with CIDP: the Privigen Post Approval Safety Study
Amgad Shebl
- 8.00 Intravenous immunoglobulin in CIDP (IOC trial)
Filip Eftimov
- 8.05 Optimal treatment in CIDP (OPTIC trial)
Filip Eftimov
- 8.10 INCbase CIDP registry: Outcome measures for clinical trials
Filip Eftimov
- 8.15 Efficacy and Safety of Three Different Dosages of IVIG (PANZYGA®) in Patients with CIDP (ProCID Study): Design of a Phase 3 Study
David Cornblath
- 8.20 Issues in Clinical Trials Designs
Deborah Gelinas
- 8.25 Closing & remarks

TUESDAY 11 JULY 2017

7.30 – 8.30 **Clinical Trial Updates** AUDITORIUM **CMTR Day 2**

Chairs: **David R. Cornblath** and **Mary M. Reilly**

- 7.30 Treating Hereditary Sensory and Autonomic Neuropathy Type 1 (HSAN1) with L-Serine
Vera Fridman
- 7.40 Aerobic Exercise for Subjects Affected by CMT Neuropathy: Results of a Multicenter, Prospective, Randomized, Single Blind, Controlled Clinical Trial
Angelo Schenone
- 7.50 A Phase 3 multi-center, randomised, double-blind, placebo controlled trial to assess the efficacy and safety of PXT3003 for CMT1A (PLEO-CMT)
René Goedkoop
- 7.55 A Phase 2 Study of ACE-083 in Patients with CMT1 or CMTX and Ankle Dorsiflexion Weakness
Kenneth M. Attie
- 8.00 Characteristic of Recovery from Muscle Fatigue in CMT Patients with Electromyographic Study (Third Report)
Toshinori Shimoi
- 8.05 Announcement of IFB-088 clinical trial for CMT1A patients
Caroline Treins
- 8.10 Closing & remarks

WEDNESDAY 12 JULY 2017

7.30 – 8.30 **Clinical Trial Updates** AUDITORIUM **GBS and Neuropathy**

Chairs: **David R. Cornblath** and **Mary M. Reilly**

- 7.30 Efficacy and safety of eculizumab in patients with Guillain-Barre syndrome: a randomized, double blind, placebo-control, phase 2 trial (JET-GBS)
Satoshi Kuwabara
- 7.45 Complement-related biomarkers in GBS: Analysis of IGOS serum and CSF samples
Ted Yednock
- 7.55 Small volume plasma exchange for Guillain-Barré syndrome in low-income countries: a safety and feasibility study
Badrul Islam
- 8.05 Second IVIg Dose in Guillain-Barre syndrome patients with poor prognosis; an update.
Christa Walgaard
- 8.10 Patient Assisted Intervention for Neuropathy: Comparison of Treatment in Real Life Situations (PAIN-CONTRoLS)
Richard J. Barohn
- 8.15 Pregabalin for neuropathic pain in Epidermolysis Bullosa
Margarita Calvo
- 8.20 Ixazomib, Lenalidomide, and Dexamethasone for Patients with POEMS Syndrome
Michelle Mauermann
- 8.25 Closing & remarks