

# 2019 ANNUAL MEETING GENOA, ITALY • 22-25 JUNE

CENTRO CONGRESSI - PORTO ANTICO DI GENOVA



# PROGRAM



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# Program

**2019  
PNS Annual Meeting**

**22-25 June  
Genoa, Italy**

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On behalf of the Peripheral Nerve Society, please let me welcome you to the 2019 Annual Meeting at the Centro Congressi – Porto Antico di Genova, in Genoa, Italy, 22–25 June 2019.

The PNS Annual Meeting continues to be the premier venue for learning about advances in peripheral neuropathy and peripheral nerve biology. The Program Committee has organized a spectacular meeting: the traditional mixture of carefully selected plenary lectures, oral platforms, oral posters, and poster sessions, as well as expanded symposia that were organized by the special interest groups — Charcot-Marie-Tooth and Related Neuropathies Consortium (CMTR), the Inflammatory Neuropathy Consortium (INC), the International Diabetes Neuropathy Consortium (IDNC), and the Toxic Neuropathy Consortium (TNC). We will also continue the innovative clinical trials session on Tuesday afternoon.



The local organizing committee and the PNS team have worked hard to enable the PNS to enjoy the beautiful and historic city of Genoa.

- On Saturday night, please join us for the Opening Reception at the Carlo Felice Theatre. The reception will feature classical music performed by the Genoa music conservatory, traditional Italian food, and drinks.
- On Sunday night, all are invited, as we honor Junior and new members of the Society with a reception at the Aquarium of Genoa. Sponsored by Akcea, this reception will feature guided exhibits, food, and beverages.
- On Tuesday night, please join us for a spectacular PNS Closing Reception at the beautiful Villa Lo Zerbindo. Transportation will be provided to and from the Convention Centre. This event should not be missed!

Coffee breaks and lunch will be provided daily for delegates. Please see the program for additional details. Complimentary WiFi will also be provided (instructions can be found on page 12 of the program).

All are encouraged to attend the PNS Annual General Meeting on Monday, 24 June at 12.30. Your input is valuable, as we review the business of the PNS.

I want to personally thank our sponsors, the Scientific Program Committee, PNS Board, meeting faculty, staff, and everyone who has generously donated their time and efforts to put together this outstanding meeting. We sincerely hope you enjoy the 2019 PNS Annual Meeting and your stay in Italy.

A handwritten signature in black ink that reads "Steven Scherer". The signature is fluid and cursive.

Steven S. Scherer, MD, PhD  
President, Peripheral Nerve Society

# PNS LEADERSHIP AND PROGRAM ORGANIZERS

## OFFICERS

**Steven S. Scherer, MD, PhD**, President, *The University of Pennsylvania, Philadelphia, PA, USA*  
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**Mary M. Reilly, MD, FRCP, FRCPI**, Past President, *UCL Institute of Neurology, London, UK*

## BOARD MEMBERS

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**Alessandra Bolino, PhD**, Co-Chair, *Scientific Program Committee, San Raffaele Hospital, Milan, Italy*  
**Chiara Briani, MD**, *University of Padova, Padova, Italy*  
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**Kazim Sheikh, MD**, *McGovern Medical School, Houston, TX, USA*

## INFLAMMATORY NEUROPATHY CONSORTIUM REPRESENTATIVE

**Richard Lewis, MD**, *Cedars-Sinai, Los Angeles, CA, USA*

## CHARCOT-MARIE-TOOTH AND RELATED NEUROPATHIES CONSORTIUM REPRESENTATIVE

**Davide Pareyson, MD**, *Carlo Besta Neurologic Institute, Milan, Italy*

## TOXIC NEUROPATHY CONSORTIUM REPRESENTATIVE

**Guido Cavaletti, MD**, *University of Milano-Bicocca, Monza (I), Milan, Italy*

## INTERNATIONAL DIABETES NEUROPATHY CONSORTIUM REPRESENTATIVE

**Christopher Gibbons, MD**, *Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA*

## JPNS EDITOR

**David Cornblath, MD**, *Johns Hopkins University, Baltimore, MD, USA*

## SCIENTIFIC PROGRAM COMMITTEE

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**John Svaren, PhD**, Co-Chair, *University of Wisconsin, Madison, Wisconsin, USA*  
**Chiara Briani, MD**, *University of Padova, Padova, Italy*  
**Guido Cavaletti, MD**, *University of Milano-Bicocca, Monza (I), Milan, Italy*  
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**Christopher Gibbons, MD**, *Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA*  
**Ahmet Hoke, MD, PhD**, *Johns Hopkins Medicine, Baltimore, MD, USA*  
**Giuseppe Lauria, MD**, *Carlo Besta Neurologic Institute, Milan, Italy*  
**Davide Pareyson, MD**, *Carlo Besta Neurologic Institute, Milan, Italy*  
**Amanda Peltier, MD**, *Vanderbilt University, Brentwood, TN, USA*  
**Michael Polydefkis, MD, MHS**, *Johns Hopkins Medicine, Baltimore, MD, USA*  
**Luis Querol, MD, PhD**, *Hospital de Sant Pau, Barcelona, Spain*  
**Mary Reilly, MD**, *UCL Institute of Neurology, London, UK*  
**Steven Scherer, MD, PhD**, *The University of Pennsylvania, Philadelphia, PA, USA*  
**Nortina Shahrizaila, PhD**, *University of Malaya, Kuala Lumpur, Malaysia*  
**Gordon Smith, MD, FAAN**, *Virginia Commonwealth University, Richmond, VA, USA*  
**Charlotte Sumner, MD**, *John Hopkins Medicine, Baltimore, MD, USA*  
**Pedro Tomaselli, MSc**, *Clinical Hospital of Ribeirão Preto, University of São Paulo, São Paulo, Brazil*  
**Pieter Van Doorn, MD, PhD**, *Erasmus MC University Medical Center, Rotterdam, Netherlands*

# PNS LEADERSHIP AND PROGRAM ORGANIZERS

## LOCAL ORGANIZING COMMITTEE

**Eduardo Nobile-Orazio, MD, PhD**, Co-Chair, *Milan University, Milan, Italy*  
**Angelo Schenone, PhD**, Co-Chair, *University of Genoa, Italy*  
**Luana Benedetti, MD**, *University of Genoa, Genoa, Italy*  
**Marina Grandis, MD**, *University of Genoa, Genoa, Italy*  
**Gianluigi Mancardi, PhD**, *President, Italian Society of Neurology, Genoa, Italy*  
**Lucilla Nobbio**, *University of Genoa, Genoa, Italy*

## JUNIOR COMMITTEE

**Vera Fridman, MD**, Chair, *University of Colorado Anschutz Medical Campus, Aurora, CO, USA*  
**Pedro Tomaselli, MSc**, Chair-Elect, *Clinical Hospital of Ribeirão Preto, São Paulo, Brazil*  
**Cervellini, Oxford, PhD**, *University of Oxford, Oxford, UK*  
**Rhona McGonigal, PhD**, *University of Glasgow, Glasgow, UK*  
**Oranne Sanmaneechai, MD**, *Siriraj Hospital, Mahidol University, Bangkok, Thailand*  
**Amro Stino, MD**, *Ohio State University, Columbus, OH, USA*  
**Yuzhong Wang, MD**, *Affiliated Hospital of Jining Medical University, Jining, China*

## PRIZE COMMITTEE

**Giuseppe Lauria, MD**, Co-Chair, *IRCCS "Carlo Besta" Neurological Institute & University of Milan, Milan, Italy*  
**Amanda Peltier, MD**, Co-Chair, *Vanderbilt University Medical Center, Brentwood, TN, USA*  
**Catharina Faber, MD, PhD**, *Maastricht University Medical Centre, Maastricht, Netherlands*  
**Shawna Feely, MS, CGC**, *University of Iowa, Iowa City, IA, USA*  
**Ahmet Hoke, MD, PhD**, *Johns Hopkins University, Baltimore, MD, USA*  
**Ruth Huizinga, PhD**, *Erasmus MC, Rotterdam, Netherlands*  
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**Susanna Park, PhD**, *University of Sydney, Sydney, Australia*  
**Luis Querol, MD, PhD**, *Hospital de la Santa Creu | Sant Pau Barcelona, Spain*  
**James Russell, MD, MS**, *University of Maryland School of Medicine, Baltimore, MD, USA*

# MEMBERSHIP INFORMATION



## **EDUCATION. ADVOCACY. NETWORKING. PROFESSIONAL DEVELOPMENT.**

The Peripheral Nerve Society provides Annual Meetings, Teaching Courses, Guidelines, and other resources to aid in the education of members. Becoming a member of PNS means collaborating with prominent global professionals in the field to develop and provide the best treatments for people with peripheral nerve diseases and setting standards of care within the field.

Membership in the Peripheral Nerve Society is open to any professional with interest in disorders of the peripheral nervous system. Members of the Peripheral Nerve Society may join the Inflammatory Neuropathy Consortium, International Diabetes Consortium, Toxic Neuropathy Consortium and Charcot-Marie-Tooth and Related Neuropathy Consortium by request. Our membership includes clinicians, academicians, basic and clinical scientists, members of industry and representatives from neuropathy organizations. All members receive a subscription to the Journal of the Peripheral Nervous System (JPNS) and a discounted registration to our Annual Meeting.

PNS Membership Features Include:

- Subscription to Journal of the Peripheral Nervous System (JPNS)
- Discounted PNS Annual Meeting Rates
- Access to Member Directory
- Patient Information
- Networking Opportunities
- Career Planning
- Volunteer Opportunities
- Voting Rights
- PNS Members may join special interest groups upon request (IDNC, INC, TNC, or CMTR)

Visit [www.PNSociety.com](http://www.PNSociety.com) to join today!

## ABOUT THE SOCIETY

The Peripheral Nerve Society (PNS) was founded in 1994 from two groups of academic investigators, Peripheral Nerve Study Group and Peripheral Neuropathy Association of America, interested in the basic biology and function of the peripheral nervous system and its application to the clinic. Their invite-only, biennial meetings involved 80-125 attendees in cloistered settings organized by shoestring and local initiative. From this, we have grown remarkably. We now have an annual meeting of over 600 people including meetings within the meeting for the special interest groups in inflammatory, diabetic, toxic, and hereditary neuropathy. With this substantial growth and the success of JPNS, the Journal of the Peripheral Nervous System, the Society continues to flourish.

The Peripheral Nerve Society provides Annual Meetings, Teaching Courses, Guidelines, and other resources to aid in the education of members. Becoming a member of PNS means collaborating with prominent global professionals in the field to develop and provide the best treatments for people with peripheral nerve diseases and setting standards of care within the field. Please participate in our future by joining the PNS, volunteering for a project aligned with your interests and sending your ideas for the future to the Executive Office, or Board member.

### CONTACT US

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## PNS EXECUTIVE OFFICE STAFF



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# PAST MEETINGS

## PAST MEETINGS OF THE PNS

- 2018 Baltimore, MD, USA
- 2017 Sitges-Barcelona, Spain
- 2015 Quebec City, Canada
- 2013 Saint-Malo, France
- 2011 Potomac, MD, USA
- 2009 Würzburg, Germany
- 2007 Snowbird, UT, USA
- 2005 Tuscany, Italy
- 2003 Banff, Canada
- 2001 Tyrol, Austria
- 1999 La Jolla, CA, USA
- 1997 Cambridge, UK
- 1995 Antalya, Turkey
- 1994 Saint Paul, MN, USA

## PAST MEETINGS OF THE PNSG

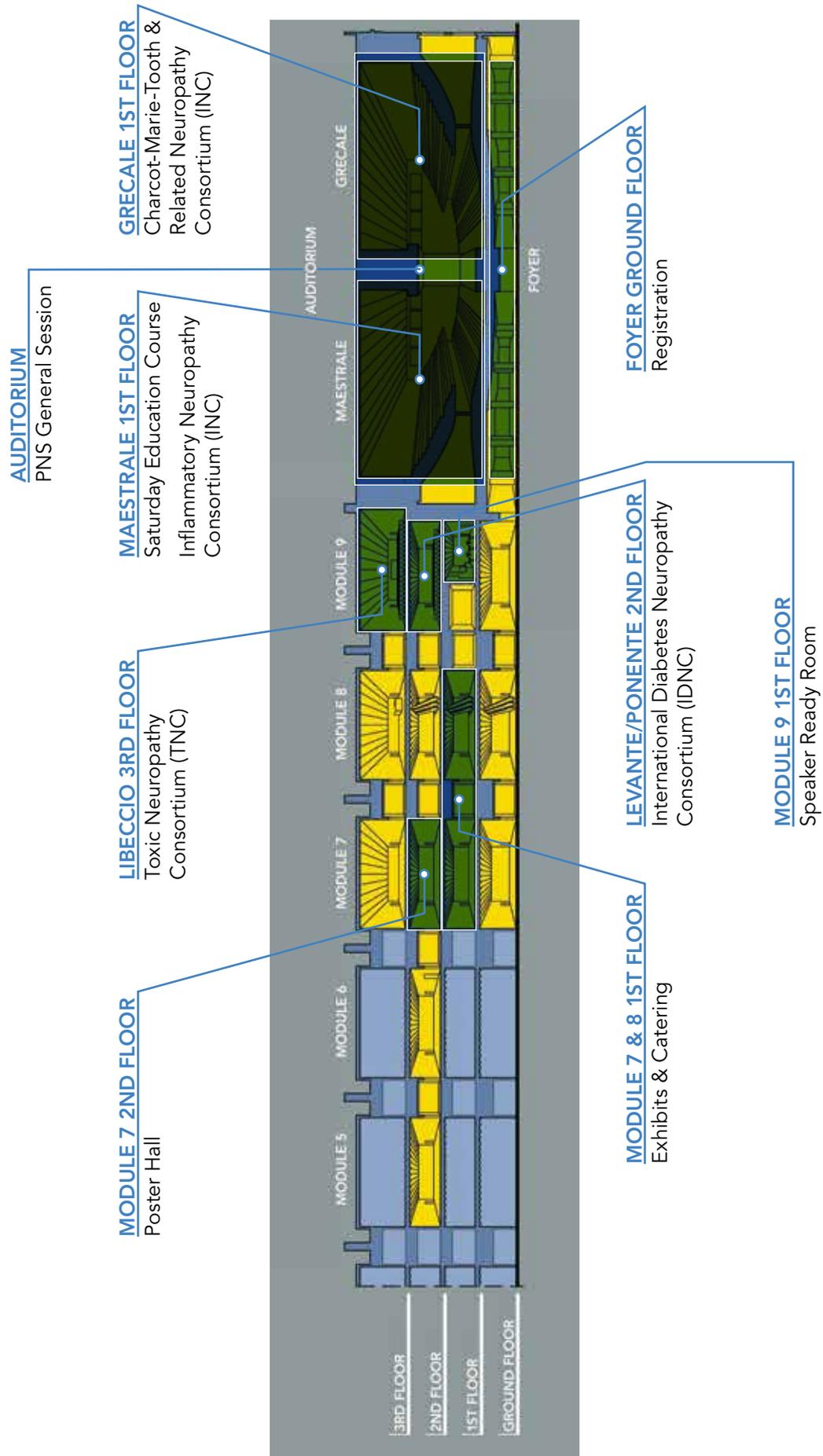
- 1993 Boppard, Germany
- 1991 Arden House, NY, USA
- 1989 Padua, Italy
- 1987 Lake Couchichinh, Ontario, Canada
- 1985 Mürren, Switzerland
- 1983 Fontevraud, France
- 1981 Shakertown, KY, USA
- 1979 Wye College, Kent, UK
- 1977 Airlie House, VA, USA
- 1975 Rochester, MN, USA
- 1974 Carville, LA, USA

## PAST MEETINGS OF THE PNA AND PNA

- 1992 Rapallo, Italy
- 1990 Oxford, UK
- 1989 Maui, HI, USA
- 1988 Halifax, Nova Scotia, Canada
- 1986 Hilton Head Island, SC, USA
- 1985 Keystone, CO, USA
- 1984 Keystone, CO, USA

# CONVENTION CENTRE FLOOR PLAN

## CENTRO CONGRESSI – PORTO ANTICO DI GENOVA



# PROGRAM AT A GLANCE

## SATURDAY 22 JUNE 2019

- 9.30-11.00 Coffee & Registration [GROUND FLOOR LOBBY](#)
- 11.00-19.00 **Education Course** [MAESTRALE](#)
- 11.00 **Welcome Address**  
Steven Scherer
- 11.10 **The Autonomic Nervous System – A Case-based Approach to Functional Neuroanatomy**  
Roy Freeman
- 11.50 **A Primer on WES, GWAS and WGS as Tools for Unravelling the Genetics of Inherited Neuropathies**  
Marina Kennerson
- 12.30 **Imaging of PNS**  
Simonetta Gerevini
- 13.10 Career Development Lunch  
[GROUND FLOOR LOBBY](#)
- 14.30 **Mammalian Models of Neuropathy – When and How to Use Them**  
Steve Scherer
- 15.10 **Emerging Therapies in Autoimmune Neuropathy – Beyond IVIg**  
Michael Lunn
- 15.50 **Neuropathy in Resource Limited Settings, What and How to Investigate**  
Umapathi Thirugnanam
- 16.30 **Educational Committee and Strategies: How to Apply for Educational Event Funding**  
Hugh Willison and Alessandra Bolino
- 16.45-17.15 Coffee Break & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 17.15-18.45 **Clinical Case Presentations** [MAESTRALE](#)
- 19.00-23.00 **Welcome Reception** [CARLO FELICE THEATRE](#)

## SUNDAY 23 JUNE 2019

- 7.15-8.15 Coffee & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 7.15-8.15 Poster Viewing [MODULE 7 2ND FLOOR](#)
- 7.15-8.15 **Sponsored Symposia**  
Kedrion Symposium [AUDITORIUM](#)  
Pfizer Symposium [LEVANTE/PONENTE](#)
- 8.30-9.00 **Peter J Dyck Lecture** [AUDITORIUM](#)  
**Neurological Disturbances in Patients with Diabetes Beyond Peripheral Neuropathy: CNS Involvement on Multimodal Brain Imaging**  
Geert Jan Biessels
- 9.00-10.30 **Platform Session I** [AUDITORIUM](#)
- 10.30-11.00 Coffee Break & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 11.00-11.30 **Richard Bunge Lecture** [AUDITORIUM](#)  
**Peripheral Nerve Homeostasis and Regeneration – Links to Disease**  
Alison Lloyd
- 11.30-12.30 **Oral Poster Session I** [AUDITORIUM](#)
- 12.30-14.30 Lunch & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 12.30-14.30 Poster Session I [MODULE 7 2ND FLOOR](#)

## 14.30-18.30 Special Interest Group (SIG) Parallel Sessions

### INFLAMMATORY NEUROPATHY CONSORTIUM (INC) [MAESTRALE](#)

- 14.30 **Monoclonal Gammopathies of Neurological Significance**  
Invited Speaker: Shirley D'Sa
- 15.00 **INC Project Updates**
- 15.30 **Oral Abstracts**
- 16.00 **Peripheral Neuropathy and Gastrointestinal Disease**  
Francisco de Assis Gondim
- 16.30 Coffee Break & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 17.00 **An Overview of Inflammatory Neuropathies and Diabetes**  
Yusuf Rajabally
- 17.30 **Oral Abstracts**

### CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM (CMTR) [GRECALE](#)

- 14.30 **Platform Presentations: Schwann Cells and Myelination**
- 15.45 **Oral Posters: iPSCs**
- 16.00 **Pathogenetic Inflammation in Models for Schwann Cell-Related Charcot-Marie-Tooth Disorders**  
Rudolf Martini
- 16.30 Coffee Break & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 17.00 **Mechanisms of TRPV4-induced Peripheral Nerve Disease**  
Charlotte Sumner
- 17.30 **Platform Presentations: Pathomechanisms**
- 18.00 **Oral Posters: Genetics and Phenotypes**

### INTERNATIONAL DIABETES NEUROPATHY CONSORTIUM (IDNC) [LEVANTE/PONENTE](#)

- 14.30 **Risk Factors for Presence and Progression of CAN in Type 2 Diabetes**  
Signe Toft Andersen
- 15.00 **Seeing What They are Feeling – The Central Nervous System in Diabetic Peripheral Neuropathy**  
Dinesh Selvarajah
- 15.30 **Deep Phenotyping to Explore Differences Between Painful and Painless Diabetic Neuropathy**  
Andreas Themistocleous
- 16.00 **Sodium Channel Mutations in Small Fiber Neuropathy: Implications for Diabetic Neuropathy**  
Daniele Cazzato
- 16.30 Coffee Break & Exhibits [MODULE 8 & 7 1ST FLOOR](#)
- 17.00 **Oral Poster Session**
- 17.45 **Emerging Concepts in the Early Detection and Management of Diabetic Neuropathy**  
Invited Speaker: Dan Ziegler

## 19.00-20.00 Sponsored Symposia

- CSL Behring Symposium [MAESTRALE](#)  
Alnylam Symposium [GRECALE](#)

## 20.10-23.00 Junior Reception [AQUARIUM](#) Sponsored by Akcea

# PROGRAM AT A GLANCE

## MONDAY 24 JUNE 2019

- 7.15-8.15 Coffee & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 7.15-8.15 Poster Viewing **MODULE 7 2ND FLOOR**
- 7.15-8.15 **Sponsored Symposia**
- Takeda Symposium **AUDITORIUM**
- Pharnext Symposium **LEVANTE/PONENTE**
- 8.30-9.00 **Arthur K Asbury Lecture** **AUDITORIUM**  
**Skin for Neurologists**  
Giuseppe Lauria
- 9.00-10.30 **Platform Session II** **AUDITORIUM**
- 10.30-11.00 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 11.00-11.30 **Jack Griffin Lecture** **AUDITORIUM**  
**Neuronal and Glial Mechanisms Regulating Nerve Regeneration**  
Valeria Cavalli
- 11.30-12.30 **Oral Poster Session II** **AUDITORIUM**
- 12.30-13.00 **PNS Annual General Meeting**  
(all are welcome) **AUDITORIUM**
- 13.00-14.30 Lunch & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 13.00-14.30 Poster Session II **MODULE 7 2ND FLOOR**
- 14.30-18.30 **Special Interest Group (SIG) Parallel Sessions**

### INFLAMMATORY NEUROPATHY CONSORTIUM (INC) **MAESTRALE**

- 14.30 **INC Project Updates**
- 15.00 **Ultrasound and MRI of Peripheral Nervous System: Usefulness for Diagnosis and Therapeutic Indication**  
Luca Padua and Simonetta Gerevini
- 16.00 **Oral Abstracts**
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Neuropathology of Inflammatory Neuropathies**  
Haruki Koike
- 17.30 **Oral Abstracts**

### CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM (CMTR) **GRECALE**

- 14.30 **Rare and Genetically Undiagnosed Forms of Inherited Peripheral Neuropathies**  
Invited Speaker: Jan Senderek
- 15.00 **Platform Presentations: Genetics, Biomarkers, Amyloidosis**
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Measuring Disability of Infants, Children and Adults with CMT**  
Joshua Burns
- 17.30 **Platform Presentations: Therapy**
- 18.15 **Oral Posters: Therapy**

### INTERNATIONAL DIABETES NEUROPATHY CONSORTIUM (IDNC) **LEVANTE/PONENTE**

- 14.30 **Ultrasound of Peripheral Nerve in Diabetes**  
Stefan Meng
- 15.00 **MR Neurography in Peripheral Neuropathies**  
Jennifer Kollmer
- 15.30 **Testing Autonomic Outcomes in Diabetic Neuropathy Research: Challenges and Advantages**  
James Russell

- 16.00 **Scientific Pitfalls in Emerging Technology for Study of Neuropathy**  
Brian Callaghan & Chris Gibbons
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Oral Poster Session**
- 17.30 **Platform Session**

### TOXIC NEUROPATHY CONSORTIUM (TNC) **LIBECCIO**

- 14.30 **Summary/Update of Activities**  
Guido Cavaletti
- 15.00 **The Power of Neurophysiology in Chemotherapy-Induced Peripheral Neuropathy**  
Susanna Park
- 15.30 **Oral Abstracts**
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Oral Abstracts**
- 17.30 **Relevance of Peripheral Neuropathy in Cancer Patients Treated with Chemotherapy**  
Alberto Sobrero
- 18:00 **Palliative Care in Oncology and Neuropathic Pain**  
Invited Speaker: Augusto Caraceni

### 19.00-20.00 **Sponsored Symposia**

- Grifols Symposium **MAESTRALE**
- Akcea Symposium **GRECALE**

## TUESDAY 25 JUNE 2019

- 7.15-8.15 Coffee & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 7.15-8.15 Poster Viewing **MODULE 7 2ND FLOOR**
- 7.15-8.15 **Sponsored Symposia**
- LFB Symposium **AUDITORIUM**
- 8.30-9.00 **PK Thomas Lecture** **AUDITORIUM**  
**Familial Amyloid Polyneuropathy as a Model of Medical Progress**  
David Adams
- 9.00-10.30 **Platform Session III** **AUDITORIUM**
- 10.30-11.00 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 11.00-11.30 **Pembroke Lecture** **AUDITORIUM**  
**The Impact of Mitochondrial Biology on Central and Peripheral Nervous System Disorders**  
Massimo Zeviani
- 11.30-12.30 **Oral Poster Session III** **AUDITORIUM**
- 12.30-14.30 Lunch & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 12.30-14.30 Poster Session III **MODULE 7 2ND FLOOR**
- 14.30-16.30 **Clinical Trial Updates** **AUDITORIUM**
- 16.30-17.00 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00-18.00 **Presidential Lecture** **AUDITORIUM**  
**Peripheral Neuropathy – 6th Edition**  
Steven Scherer
- 18.00-18.30 **Awards & Prizes** **AUDITORIUM**
- 18.30-23.00 **Closing Reception** **VILLA LO ZERBINO**  
Buses depart from convention centre main entrance every 30 minutes

# GENERAL INFORMATION

## REGISTRATION/HELP DESK HOURS

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Saturday, 22 June	9.30-18.00
Sunday, 23 June	7.00-18.00
Monday, 24 June	7.00-18.00
Tuesday, 25 June	7.00-8.00

\*The Convention Centre opens at 7.00.

## REGISTRATION FEES

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Registration fees include:

- Saturday Education Course
- Three full days of regular programming with special interest group afternoon parallel sessions
- Three exciting off-site receptions
- Access to sponsor marketplace and exhibitor directory
- A chance to network with friends, new and old
- Lunch and coffee breaks

## BADGE

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All conference participants are required to wear their name badge at all conference functions.

## CERTIFICATE OF ATTENDANCE

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Your certificate will be emailed to you after the meeting.

## LIABILITY & INSURANCE

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The organizers accept no responsibility for any injury or damage involving persons and property during the 2019 PNS Meeting.

## SPEAKER READY ROOM – MODULE 9 (1ST FLOOR)

---

All speakers must upload presentations here, at least 60 minutes prior to the session start time.

Saturday, 22 June	9.00-19.00
Sunday, 23 June	7.00-19.30
Monday, 24 June	7.00-19.30
Tuesday, 25 June	7.00-18.00

\*Note: Speaker designations are listed in the program, as submitted

## WIFI INSTRUCTIONS

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Complimentary WIFI is available throughout the convention centre.

Network: **PNSociety2019**

Password: **PNSociety2019**

## MOBILE APP INSTRUCTIONS

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Navigate the event like a pro with the PNS 2019 Annual Meeting mobile app, powered by Core-apps.com.

With the PNS 2019 Annual Meeting app, you can:

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## EXHIBITION

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Please be sure to visit the exhibits located in Module 7 & 8, 1st Floor. All catering service will be located here.

Exhibits open at 16.45 on Saturday 22 June.

## POSTER SESSIONS

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Complete abstract details and poster numbers may be found in the online supplement and the mobile app.

Poster Session I:     Sunday 23 June  
                              Room: Module 7 (2nd Floor)  
                              Mandatory Stand by Time: 12.30-14.30

Poster Session II:    Monday 24 June  
                              Room: Module 7 (2nd Floor)  
                              Mandatory Stand by Time: 13.00-14.30

Poster Session III:   Tuesday 25 June  
                              Room: Module 7 (2nd Floor)  
                              Mandatory Stand by Time: 12.30-14.30

Poster Set-Up:        17.00-19.00 the evening prior to your session  
Poster Dismantle:    14.30-16.00 the day of your session

# ACKNOWLEDGEMENTS

*We thank the generosity of our sponsors.  
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## PATIENT ADVOCACY GROUPS (PAGS)

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*Please reference page 50 for the exhibitor directory.*

# INDIVIDUAL MEETINGS

## SATURDAY 22 JUNE 2019

- 8.00-10.45 Inherited Neuropathy Consortium Meeting **LIBECCIO: MODULE 9 – 3RD FLOOR**  
*This is an open meeting. All are welcome to attend.*
- 9.00-11.00 IGOS Meeting **ZEFIRO: MODULE 9 – 2ND FLOOR**  
*This is a closed meeting.*
- 11.15-12.45 INCBASE **LIBECCIO: MODULE 9 – 3RD FLOOR**  
*This is an open meeting. All are welcome to attend.*
- 13.00-14.00 Patient Advocacy Group Meeting **BORA: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*
- 14.00-15.30 International Diabetes Neuropathy Consortium Board Meeting **MARIN: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*
- 14.00-16.30 CMTPed's Clinical Evaluator Meeting **ZEFIRO: MODULE 9 – 2ND FLOOR**  
*This is a closed meeting.*
- 16.00-17.00 Toxic Neuropathy Consortium Board Meeting **BORA: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*
- 16.00-17.00 Charcot-Marie-Tooth & Related Neuropathies Board Meeting **MARIN: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*
- 16.00-17.00 Organizing a PNS Basic Science Consortium **AUSTRO: MODULE 9 – 1ST FLOOR**  
*This is an open meeting. All are welcome to attend.*
- 17.00-18.00 CMTR Genetic Consortium Working Group **MARIN: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*
- 17.00-18.30 Vasculitic Neuropathy Study Group **BORA: MODULE 9 – 1ST FLOOR**  
*This is a closed meeting.*

## SUNDAY 23 JUNE 2019

- 10.30-11.00 Top Sponsors Meeting **VIP ROOM\***  
*This is a closed meeting.*

## MONDAY 24 JUNE 2019

- 10.30-11.00 International Congress on Neuromuscular Diseases (ICNMD) Education Meeting **VIP ROOM\***  
*This is a closed meeting.*
- 12.00-13.00 Peripheral Nerve Society Education Committee **VIP ROOM\***  
*This is a closed meeting.*

## TUESDAY 25 JUNE 2019

- 10.30-11.00 2020 Program Committee Meeting **VIP ROOM\***  
*This is a closed meeting.*
- 12.45-13.45 Prize Committee Meeting **VIP ROOM\***  
*This is a closed meeting.*
- 16.30-17.00 Prize Committee Meeting **VIP ROOM\***  
*This is a closed meeting.*

## WEDNESDAY 26 JUNE 2019

- 7.30-8.30 Inflammatory Neuropathy Consortium Board Meeting **OCEANO ROOM, NH MARINA HOTEL**  
*This is a closed meeting.*
- 9.00-12.00 Peripheral Nerve Society Board Meeting **OCEANO ROOM, NH MARINA HOTEL**  
*This is a closed meeting.*

\*VIP room is located in the foyer Maestrale on the ground floor, behind the escalators.

A large, ornate fountain in a city square, likely in Bucharest, Romania. The fountain features multiple tiers and numerous water jets. In the background, there are grand, classical-style buildings with domes and arched windows. The sky is bright blue with scattered white clouds. A semi-transparent blue horizontal band is overlaid across the middle of the image, containing the word "Schedule" in white text.

# Schedule

## PROGRAM – SATURDAY 22 JUNE 2019

9.30-11.00 Coffee & Registration **GROUND FLOOR LOBBY**11.00-13.10 **Education Course MAESTRALE**

**Chairs: Vera Fridman, MD, University of Colorado Anschutz Medical Campus, Aurora, CO, USA; Matilde Laura, MD, PhD, MRC Centre for Neuromuscular Diseases, UCL Institute of Neurology, London, UK**

11.00 **Welcome Address**

**Steven Scherer, MD, PhD, The University of Pennsylvania, Philadelphia, PA, USA**

11.10 **The Autonomic Nervous System – A Case-based Approach to Functional Neuroanatomy**

**Roy Freeman, MBChB, Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA**

Autonomic nerve fibers are small, lightly myelinated and unmyelinated and are injured to a varying degree in most symmetrical generalized, peripheral neuropathies. While this injury is often mild or subclinical, there are a group of peripheral neuropathies in which the small or unmyelinated fibres are selectively or prominently targeted. These include the autonomic neuropathies associated with diabetes and amyloid; immune-mediated autonomic neuropathies including those associated with a paraneoplastic syndrome; inherited autonomic neuropathies; autonomic neuropathies associated with infectious diseases and toxic autonomic neuropathies. In some peripheral neuropathies, the autonomic features are the most prominent manifestation of the neuropathy, while, in others, autonomic manifestations may be less prominent compared to the sensory or motor manifestations. This lecture will adopt a case-based approach to illustrate the functional anatomy and physiology of the autonomic nervous system.

11.50 **A Primer on WES, GWA and WGS as Tools for Unravelling the Genetics of Inherited Neuropathies**

**Marina Kennerson, PhD, ANZAC Research Institute, Sydney, Australia**

Identifying new genes for inherited peripheral neuropathies (IPN) has flourished since the introduction of next generation sequencing with over 85 causative genes for CMT and related disorders reported to date. Despite this success up to 40% of families remain genetically undiagnosed. Are more genes to be identified? Can we target genes explaining the variability of the IPN phenotypes? Do non-coding mutations contribute to the unsolved IPN families? This presentation will give a primer on high throughput genomics (array genotyping, whole exome/genome sequencing) and their use with traditional gene mapping methods (positional cloning and genome wide association studies; GWAS) to further understand the genetics of IPNs. The principles of Mendelian WES variant filtering, GWAS studies to identify modifier genes and WGS analysis to identify structural variation mutations will be outlined along with the respective strengths and challenges of these approaches.

12.30



### Imaging of Peripheral Nervous System

**Simonetta Gerevini, MD, IRCCS Ospedale San Raffaele, Milano, Italy**

Magnetic resonance imaging provides a useful tool that can be used in the diagnosis of peripheral nerve disorders. The lecture will cover what aspects can be visualized with MR imaging and describe the relationship of signal alteration and morphologic modification of nerves and muscles to disorders of the peripheral nervous system. Examples of techniques and findings in inflammatory neuropathies, genetic neuropathies and pudendal neuropathy will be provided.

13.10-14.30

Career Development Lunch **GROUND FLOOR LOBBY**

14.30-16.45

### Education Course continued **MAESTRALE**

**Chairs: Catharina Faber, MD, PhD, Maastricht University Medical Centre, Maastricht, Netherlands; Christopher Klein, MD, Mayo Clinic, Rochester, MN, USA**

14.30



### Mammalian Models of Neuropathy – When and How to Use Them

**Steven Scherer, MD, PhD, The University of Pennsylvania, Philadelphia, PA, USA**

This lecture will review animal models of neuropathy. Animals have been key for illuminating the basic biology of nerves and the pathogenesis of many kinds of neuropathies (both genetic and acquired), as well as evaluating new treatments for neuropathy. Rodents (mice and rats) have been the main models, and provide robust models of nerve injury, as well as toxic, diabetic, inflammatory, and genetic neuropathies, and as a platform for testing therapies. The ability to image living zebrafish larvae has provided insights into axonal degeneration and regeneration, and also to model toxic and genetic neuropathies. *Drosophila* and *C. elegans* have been used as models of genetic and acquired axonal neuropathies, as well as to screen for genes that play essential roles in axonal degeneration.

15.10



### Emerging Therapies in Autoimmune Neuropathy – Beyond IVIg

**Michael Lunn, FRCP PhD, National Hospital for Neurology, Queen Square, London, UK**

The autoimmune neuropathies are a diverse group of conditions. The pathogenesis of these conditions, which is varied and complex, is becoming better understood. Steroids, PLE<sub>x</sub> and IVIg, remain the backbone of treatment for these disorders. The primary conductor for an orchestra of immunological instruments varies from disease to disease. The ability to tailor treatment to interfere with the major players will help us to reduce the burden of disability and reduce adverse effects. Treatments being made available for our use, often harvested from other specialties, are becoming useful in treating disease more specifically. Anti-CD20 drugs might be useful in antibody-mediated disease, especially those with IgG4 antibodies. Where rituximab fails, tyrosine kinase and proteasome inhibitors may be important in depleting the B-cell lineage. In T-cell mediated disease drugs from MS might be helpful, and complement inhibition has shown promise, and immunomodulation in cytokine-driven disease can effect a long term remission. This lecture will briefly cover all of these concepts and emphasize the need for collaborative and well-designed trials to prove or disprove efficacy.

## PROGRAM – SATURDAY 22 JUNE 2019

15.50

**Neuropathy in Resource Limited Settings, What and How to Investigate**

**Umapathi Thiruganham, MBBS, MRCP, National Neuroscience Institute, Singapore, Singapore**

The earth is neither flat nor round, but rather a pyramid with an apex occupied by the top 10%. At the base are 4 billion people making <8 USD/day. Business, economies and, by extrapolation, medical activities largely cater to the upper sections of the pyramid. The circumstances at the bottom are unique; and the strategies employed in the apex seldom work for the base. Using illustrative cases, this lecture will present disruptive innovations to improve diagnosis and management of peripheral neuropathy at the base of the pyramid. "These unhappy times call for the building of plans that rest upon the forgotten, ...that put faith once more in the forgotten man at the bottom of the economic pyramid" – Franklin D Roosevelt.

16.30

**Educational Committee and Strategies: How to Apply for Educational Event Funding**

**Hugh Willison, MD, PhD, University of Glasgow, Glasgow, UK;**  
**Alessandra Bolino, PhD, IRCCS Ospedale San Raffaele, Milano, Italy**

16.45-17.15

Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

17.15-18.45

**Clinical Case Presentations MAESTRALE**

**Chairs: Vera Fridman, MD, University of Colorado Anschutz Medical Campus, Aurora, CO, USA; Pedro Tomaselli, MSc, Clinical Hospital of Ribeirão Preto, University of São Paulo, São Paulo, Brazil**

17.15

**A Mitochondrial ATP6 Mutation Causing a Slowly Progressive Myeloneuropathy**

**Tanya Bardakjian, MS, LCGC, University of Pennsylvania, Philadelphia, PA, USA**

17.30

**Adult Polyglucosan Body Disease Presenting with a Peripheral Neuropathy: Broadening the Clinical Spectrum**

**Jonathan De Winter, Antwerp University Hospital, Edegem, Belgium**

17.45

**Atypical Sensorimotor Neuropathy Related to Cutaneous Toxigenic Diphtheria Infection in a World Traveler**

**Penelope Spring, MBBS (Hons I), FRACP, PhD, Department of Neurology, Concord Repatriation General Hospital, Sydney, NSW, Australia**

18.00

**Mutations in SLC25A46 Cause a Spectrum of Clinical Phenotypes Including Axonal CMT with Optic Atrophy**

**Carolynne Doherty, MRC Centre for Neuromuscular Diseases, UCL Queen Square Institute of Neurology, London, UK**

## PROGRAM – SATURDAY 22 JUNE 2019

SATURDAY

- 18.15 **Rare and Challenging Case of Guillain-Barre Syndrome Associated with Stroke Secondary to Spotted Fever**  
**Anomali Vidanagamage, MBBS, MD, National Hospital, Colombo, Sri Lanka**
- 18.30 **PNS Toxicity Induced by Immune-checkpoint Inhibitors in Cancer Patients: Single Centre Experience**  
**Silvia Bocci, Department of Medicine, Surgery and Neurosciences - University of Siena, Siena, Italy**

19.00-23.00 **Welcome Reception** CARLO FELICE THEATRE



The 2019 PNS Welcome Reception will kick off at the Teatro Carlo Felice, a short walk from the Convention Centre. The theatre is the principle opera house in Genoa and is located on the Piazza De Ferrari. Guides with PNS signs will lead walking groups to the theatre from the convention centre. Please make sure to wear your badge, as this will be your ticket into the theatre. We will start the evening with opening remarks and classical music performed by members of the local music conservatory in Genoa. **Cocktails and dinner will be served on the veranda starting at 20.00.**

Transportation will be on your own as this is walking distance from the Convention Centre. Address: Passo Montale Eugenio, 4, 16121 Genova GE, Italy

## PROGRAM – SUNDAY 23 JUNE 2019

7.15-8.15 Coffee & Exhibits **MODULE 8 & 7 1ST FLOOR**

7.15-8.15 Poster Viewing **MODULE 7 2ND FLOOR**

7.15-8.15 Sponsored Symposia



### **Kedrion Symposium: Possible New Therapeutic Options for IVIG in Neurology** AUDITORIUM

**Chair: Eduardo Nobile-Orazio, MD, PhD, FAAN, Milan University, Milan, Italy; Angelo Schenone, PhD, University of Genoa, Genoa, Italy**

- 7.15 **Unmet Clinical Needs in the Prevention and Treatment of Chemotherapy-Induced Peripheral Neurotoxicity (CIPN)**  
**Andreas Argyriou, PhD, Saint Andrew's General Hospital, Patras, Greece**
- 7.30 **The Experience of IVIg intravenous immunoglobulin in the Treatment of CIPN – Preliminary Data on Animal Model**  
**Guido Cavaletti, MD, University of Milano-Bicocca, Monza (I), Monza, Italy**
- 7.45 **The Rational for the Use of IVIG intravenous immunoglobulin in Toxic Neuropathies**  
**Nathan Staff, PhD, Mayo Clinic, Rochester, MN, USA**
- 8.00 **Open Discussion (Q&A)**



### **Pfizer Symposium: ATTR (Amyloid Transthyretin) Amyloidosis: The Source, the Impact, the Management** LEVANTE/PONENTE

**Chairs: Teresa Coelho, MD, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal; Laura Obici, MD, University of Pavia, Pavia, Italy**

- 7.15 **Welcome and Introduction**  
**Teresa Coelho, MD, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal; Laura Obici, MD, University of Pavia, Pavia, Italy**
- 7.20 **TTR in Health and Disease: What Role Does It Really Play?**  
**Laura Obici, MD, University of Pavia, Pavia, Italy**
- 7.35 **Clinical Follow Up of Asymptomatic Gene Carriers: Achieving the Earliest Diagnosis and Therapeutic Intervention**  
**Isabel Conceição, MD, Centro Hospitalar Lisboa Norte, Hospital de Santa Maria, Lisboa, Portugal**
- 7.50 **The Patient Journey: Improving Care Through a Personalized Approach to Follow Up**  
**Teresa Coelho, MD, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal**
- 8.05 **Audience Q&A**  
**Teresa Coelho, MD, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal**
- 8.15 **Summary and Close**  
**Teresa Coelho, MD, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal**

8.30-9.00

**Peter J Dyck Lecture: Neurological Disturbances in Patients with Diabetes Beyond Peripheral Neuropathy: CNS Involvement on Multimodal Brain Imaging** AUDITORIUM

**Geert Jan Biessels, MD, PhD**, Department of Neurology, Brain Center Rudolf Magnus, University Medical Center Utrecht, Utrecht, Netherlands

**Chair: Christopher Gibbons, MD**, Beth Israel Deaconess Medical Center, Harvard Medical Center, Boston, MA, USA



Geert Jan Biessels is Professor of Neurology with a chair on Cerebrovascular disease and Cognition at the Brain Center Rudolf Magnus of the University Medical Centre Utrecht, the Netherlands. He obtained his PhD in 1997 and registered as a neurologist in 2004. His major research interest is vascular cognitive impairment (VCI), focusing on etiological research using novel MRI markers and on the role of diabetes. Using the unique properties of high field 7T-MRI his group has been able — for the first time — to detect cerebral microinfarcts in vivo and subsequently translated the technique to 3T-MRI. His team is currently assessing structure and function of the small vessels themselves, thus getting closer to the core of cerebral small vessel diseases (SVD). They also employ brain network analyses and lesion symptom mapping techniques in order to better understand cognitive impact of vascular brain injury. He is PI of the TRACE-VCI study, the META VCI map initiative and the Dutch Heart Brain Connection program. He participates in the EU-funded research program SVDs@target (<https://www.svds-at-target.eu/>). He is PI of two large multicentre RCTs on prevention of cognitive decline in patients with type 2 diabetes. In 2015 he received the senior investigator award of the European Stroke Organisation (ESO) and in 2016 he was appointed Fellow of the Royal College of Physicians Edinburgh in recognition of his work.

9.00-10.30

**Platform Session I** AUDITORIUM

**Chairs: Angelo Schenone, MD**, Università degli Studi di Genova, Genoa, Italy;

**Jerome Devaux, PhD**, Institut de Neurosciences de Montpellier, Montpellier, France

- 9.00 **Biallelic Neurofascin Variants Affect Paranodal Axoglial Junctions Causing Neurodevelopmental Impairment and Central and Peripheral Demyelination**  
**Stephanie Efthymiou**, UCL Queen Square Institute of Neurology, London, UK
- 9.15 **The Polygenic Architecture of Carpal Tunnel Syndrome**  
**Akira Wiberg**, University of Oxford, Oxford, UK
- 9.30 **Mutations in the Small Heat Shock Proteins HSPB1 and HSPB8 Impair the Autophagic Flux**  
**Angela Sisto**, Peripheral Neuropathy Research Group, University of Antwerp, Antwerp, Belgium
- 9.45 **Targeting a Core Axonal Degeneration Program to Treat Vincristine and Bortezomib-Induced Axonal Degeneration**  
**Stefanie Geisler, MD**, Washington University School of Medicine in St. Louis, St. Louis, MO, USA
- 10.00 **Rab35 GTPase is an Inhibitor of mTORC1 and Regulates Myelination in the Peripheral Nervous System**  
**Federica Grandi, MSc**, IRCCS Ospedale San Raffaele, Milano, Italy

## PROGRAM – SUNDAY 23 JUNE 2019

10.15 **Dominant Mutations of a Notch Pathway Component Cause Type 2 Charcot-Marie-Tooth Disease**

**Jeremy Sullivan, PhD**, Johns Hopkins University School of Medicine, Baltimore, MD, USA

10.30-11.00 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

11.00-11.30 **Richard Bunge Lecture: Peripheral Nerve Homeostasis and Regeneration – Links to Disease** AUDITORIUM

**Alison Lloyd, PhD**, MRC Laboratory for Molecular Cell Biology, University College London, London, UK

**Chair: Ahmet Hoke, MD, PhD**, Johns Hopkins University, Baltimore, MD, USA



Alison Lloyd is a Professor of Cell Biology and head of the Cell Biogenesis and Tissue Regeneration research group at the MRC Laboratory for Molecular Cell Biology at University College London, UK. She studied Biochemistry for her undergraduate studies at UCL, followed by a PhD in the laboratory of Chris Marshall at the Chester Beatty Laboratories at the Institute of Cancer Research in London. This was followed by postdoctoral fellowships in Strasbourg, France and at the ICRF laboratories in Lincoln's Inn Fields in London. In 1998, she started her laboratory at the MRC Laboratory for Molecular Cell Biology where she currently runs a research group focused on two main areas of cell biology — the control of cell biogenesis and tissue regeneration — and how these relate to tumour formation. Using the mammalian PNS as a model system, she has made ground-breaking discoveries as to how this tissue regenerates and the parallels to the development and spread of tumours- in particular to tumours that develop in patients with the familial tumour predisposition syndrome, Neurofibromatosis Type 1. She is a member of EMBO and co-chair of the UCL Cancer Domain.

11.30-12.30 **Oral Poster Session I** AUDITORIUM

**Chairs: Oranee Sanmaneechai, MD**, Siriraj Hospital, Mahidol University, Bangkok, Thailand; **Kleopas Kleopa, MD**, The Cyprus Institute of Neurology and Genetics, Nicosia, Cyprus

11.30 **Topiramate Prevents Oxaliplatin Neurotoxicity in a Rat Model**  
**Paola Alberti, MD, PhD**, University Of Milano-Bicocca, Monza, Italy

11.35 **PRDM12 is Required for Initiation of the Nociceptive Neuron Lineage During Neurogenesis**  
**Luca Bartesaghi, PhD**, Karolinska Institute, Stockholm, Sweden

11.40 **MRI and Triple Stimulation Technique to Detect Brachial Plexus Abnormalities in Multifocal Motor Neuropathy**  
**Emilien Delmont, MD, PhD**, Referral Centre for Neuromuscular diseases and ALS, Hôpital La Timone, Marseille, France

11.45 **Role of Supportive Diagnostic Criteria in Chronic Inflammatory Demyelinating Polyradiculoneuropathy: Data from the Italian Database**  
**Eduardo Nobile-Orazio, MD, PhD, FAAN**, Milan University, IRCCS Humanitas Clinica Institute, Milan, Italy

11.50 **Guillain-Barré Syndrome following Arboviral Infection in Northeast Brazil: A Case Series**  
**Sonja Leonhard, MD**, Erasmus University Medical Center, Rotterdam, Netherlands

## PROGRAM – SUNDAY 23 JUNE 2019

SUNDAY

- 11.55 **Unravelling the Role of Niaspan in Peripheral Nerve Regeneration**  
**Stefano Previtali, MD, PhD**, Institute of Experimental Neurology (InSpe), IRCCS Ospedale San Raffaele, Milano, Italy
- 12.00 **Neuropathy Related Genes as Possible Modifiers of PMP22 Related Neuropathies**  
**Anneke van der Kooi, MD, PhD**, Amsterdam University Medical Center, Amsterdam, Netherlands
- 12.05 **Genetic and Clinical Finding in a Cohort of 341 Chinese Patients with Charcot-Marie-Tooth Disease**  
**Ruxu Zhang**, the third Xiangya Hospital, Central South University, Changsha, Hunan, China
- 12.10 **Atypical Sensorimotor Neuropathy Related to Cutaneous Toxicogenic Diphtheria Infection in a World Traveller**  
**Penelope Spring, MBBS (Hons I), FRACP, PhD**, Department of Neurology, Concord Repatriation General Hospital, Sydney, NSW, Australia
- 12.15 **Molecular Mechanisms of Impaired Sensory Nerve Regeneration in Diabetes**  
**Sung-Tsang Hsieh, MD, PhD**, National Taiwan University Hospital, Taipei, Taiwan
- 12.30-14.30 Lunch & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 12.30-14.30 Poster Session I **MODULE 7 2ND FLOOR**
- 14.30-18.30 SPECIAL INTEREST GROUP (SIG) PARALLEL SESSIONS

### INFLAMMATORY NEUROPATHY CONSORTIUM (INC) MAESTRALE

- 14.30 **Invited Lecture: Monoclonal Gammopathies of Neurological Significance**  
**Shirley D'Sa, MD, FRCP, FRCPATH**, University College London Hospitals NHS Foundation Trust, London, UK  
**Chair: Richard Lewis, MD**, Cedars-Sinai, Los Angeles, CA, USA
- 15.00 **INC Project Updates**  
**Chair: Ken Gorson, MD**, Tufts University School for Medicine, Dove, MA, USA
- 15.00 **Updates on INC**  
**Richard Lewis, MD**, Cedars-Sinai Medical Center, Los Angeles, CA, USA
- 15.05 **Update on INCBASE**  
**Filip Eftimov, MD, PhD**, Amsterdam Neuroscience, Amsterdam UMC, Amsterdam, Netherlands; **Luis Querol, MD, PhD**, Hospital de la Santa Creu I Sant Pau, Barcelona, Spain
- 15.25 **Vasculitic Neuropathy – Registry and RODS Scale Development**  
**Robert Hadden, BM BCh, FRCP PhD**, King's College Hospital, London, UK; **Mike Collins, MD**, Medical College of Wisconsin, WI, USA

- 15.30 **Oral Abstracts**  
**Chairs: Susumu Kusunoki, MD, Kindai University Faculty of Medicine, Osaka-Syama, Japan; Emilien Delmont, MD, PhD, Centre de Reference Maladies Neuromusculaires et SLA, Hôpital La Timone Adulte, Marseille, France**
- 15.30 **The Association of Dengue Infection and Guillain-Barré Syndrome in Malaysia: A Case Control Study**  
**Cheng-Yin Tan, MRCP, University of Malaya, Kuala Lumpur, Malaysia**
- 15.40 **Medical Research Council (MRC) Scores as an Outcome Measure of Strength in Guillain-Barré Syndrome**  
**Melissa Mandarakas, BHLthSci(Hons) MPhty PhD, Department of Neurology, Erasmus MC University Medical Center, Rotterdam, Netherlands**
- 15.50 **Macrophage and Perisynaptic Schwann Cell Responses to Distal Nerve Injury in an AMAN Mouse Model**  
**Madeleine Cunningham, MRes, PhD, University of Glasgow, Glasgow, UK**
- 16.00 **Peripheral Neuropathy and Gastrointestinal Disease**  
**Francisco de Assis Gondim, MD, MSc, PhD, FAAN, Universidade Federal do Ceará, Fortaleza, State of Ceará, Brazil**  
**Chair: Bart Jacobs, MD, PhD, Erasmus MC University Medical Center, Rotterdam, Netherlands**
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **An Overview of Inflammatory Neuropathies and Diabetes**  
**Yusuf Rajabally, MD, FRCP, Aston University, Birmingham, UK**  
**Chair: Robert Hadden, BM, BCh, FRCP, PhD, King's College Hospital, London, UK**
- 17.30 **Oral Abstracts**  
**Chairs: Thomas Harbo, MD, PhD, Aarhus University Hospital, Aarhus, Denmark; Jeffrey Allen, MD, University of Minnesota, Minneapolis, MN, USA**
- 17.30 **Clinical and Serological Investigations in CIDP (Chronic inflammatory demyelinating polyneuropathy) Patients with Antibodies Against CNTN1/Caspr1 Complex**  
**Elba Pascual-Goñi, MD, Neuromuscular Diseases Unit, Hospital de la Santa Creu i Sant Pau, Universitat Autònoma de Barcelona, Barcelona, Spain**
- 17.40 **Treatment Status Following Corticosteroid and Immunoglobulin Treatment in the International CIDP Outcome Study (ICOS)**  
**Sander Bus, MD, Amsterdam UMC, University of Amsterdam, Department of Neurology, Amsterdam Neuroscience, Amsterdam, Netherlands**

## PROGRAM – SUNDAY 23 JUNE 2019

SUNDAY

- 17.50 **Diagnostic Delay and Work-Up of CIDP in the International CIDP Outcome Study (ICOS) cohort**  
**Carina Bunschoten**, Erasmus MC, University Medical Center, Rotterdam, Netherlands
- 18.00 **Pure Motor Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) in 17 Patients: Clinical Characteristics, Electrophysiological Study**  
**Antoine Pegat**, Hôpital Neurologique Pierre Wertheimer, Hospices Civils de Lyon, Bron, France, Lyon, France
- 18.10 **Serum Contactin-1 Levels in Chronic Inflammatory Demyelinating Polyneuropathy – A Pilot Study**  
**Luuk Wieske, MD, PhD**, Department of Neurology, Amsterdam Neuroscience, Amsterdam UMC, Amsterdam, Netherlands
- 18.20 **CIDP with Antibodies to CNTN1 is Associated with HLA-DRB1 Haplotype**  
**Cinta Lleixà**, Hospital de la Santa Creu i Sant Pau, Barcelona, Spain

### CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM (CMTR) GRECALE

**Chairs: Stefano Previtali, MD**, IRCCS Ospedale San Raffaele Milan, Italy;  
**Adriana Rebelo, MD**, University of Miami, Miami, FL, USA

- 14.30 **Platform Presentations: Schwann Cells and Myelination**
- 14.30 **Neddylation Plays a Critical Role for Formation, Maturation and Maintenance of Schwann Cell Myelin Sheaths**  
**Ashwin Woodhoo, PhD**, CIC bioGUNE, Ikerbasque Research Foundation, Derio, Spain
- 14.45 **Cell Adhesion and Choline-Dependent Metabolism in PNS Myelination**  
**Haesun Kim, PhD**, Rutgers University, Newark, NJ, USA
- 15.00 **NRG 1 Type I Dependent Autoparacrine Stimulation of Schwann Cells in Onion Bulbs of Peripheral Neuropathies**  
**Ruth Stassart, MD, PhD**, Department of Neuropathology University Clinic Leipzig, Department of Neurogenetics MPI of Experimental Medicine, Leipzig, Germany
- 15.15 **Hyperglycosylation of Myelin Protein Zero: From Pathogenesis to Therapeutic Options**  
**Marina Grandis, MD, PhD**, University of Genova, Genova, Italy, Genova, Italy
- 15.30 **Role of the ER Stress Transcription Factor XBP1 in Charcot-Marie-Tooth Disease Type 1B**  
**Thierry Touvier, PhD**, IRCCS Ospedale San Raffaele, Milan, Italy

- 15.45 **Oral Poster Session: iPSCs**
- 15.45 **Modeling Axonal Degeneration in CMT2E Using Human Motor Neurons**  
**Mario Saporta, MD, PhD, MBA**, University of Miami Miller School of Medicine, Miami, FL, USA
- 15.50 **Modelling dHMNX and CMTX6 Using Patient Derived iPSC Motor Neurons**  
**Gonzalo Perez Siles**, Northcott Neuroscience Laboratory (ANZAC Research Institute), Sydney Medical School (University of Sydney), Sydney, Australia
- 15.55 **Transplantation of Human iPSC-derived Motor Neurons for Denervation Induced Muscle Atrophy**  
**Robert Baloh, MD, PhD**, Cedars-Sinai Medical Center, Los Angeles, CA, USA
- 16.00 **Pathogenetic Inflammation in Models for Schwann Cell-related Charcot-Marie-Tooth Disorders**  
**Rudolf Martini, PhD**, University Hospital of Würzburg, Department of Neurology, Developmental Neurobiology, Würzburg, Germany  
**Chair: Michael Sereda, PhD, MD**, Max Planck Institute for Experimental Medicine, Gottingen, Germany
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Mechanisms of TRPV4-induced Peripheral Nerve Disease**  
**Charlotte Sumner, MD**, Johns Hopkins University School of Medicine, Baltimore, MD, USA  
**Chair: Vincent Timmerman, MSc, PhD**, University of Antwerp, Antwerpen, Belgium
- 17.30 **Platform Presentations: Pathomechanisms**  
**Chairs: Marina Grandis, MD**, University of Genoa, Genoa, Italy;  
**Gita Ramdharry, MD**, Queen Square Centre for Neuromuscular Diseases, London, United Kingdom
- 17.30 **CaMKII Potentiates TRPV4 Mediated Calcium Influx, Resulting in Mitochondrial Axon Transport Disruption and Neurodegeneration**  
**Brian Woolums**, Johns Hopkins University, Baltimore, MD, USA
- 17.45 **The Integrated Stress Response Contributes to Charcot-Marie-Tooth Type 2D Peripheral Neuropathy in Mice**  
**Emily Spaulding**, The Jackson Laboratory, The University of Maine, Bar Harbor, ME, USA
- 18.00 **Oral Poster Session: Genetics & Phenotypes**
- 18.00 **Mutation Burden and Oligogenic Inheritance in a Large Inherited Axonopathy Cohort**  
**Stephan Zuchner, MD, PhD**, University of Miami Miller School of Medicine, Miami, FL, USA

## PROGRAM – SUNDAY 23 JUNE 2019

SUNDAY

- 18.05 **Mutations in Cell Adhesion Molecules Belonging to the CADM Family Cause Charcot-Marie-Tooth Disease**  
**Adriana Rebelo, PhD**, John T. Macdonald Foundation  
Department of Human Genetics, University of Miami Miller School of Medicine, Miami, FL, USA
- 18.10 **Multicenter Retrospective Study in Patients with CMT1b in France: Genotype-Phenotype Correlations**  
**Marie Subreville**, CHU La Timone, Marseille, France
- 18.15 **The Italian Registry for Charcot-Marie-Tooth Disease**  
**Davide Pareyson, MD**, Fondazione IRCCS Istituto Neurologico Carlo Besta, Milan, Italy
- 18.20 **Genotype and Phenotype in Thai Children with Charcot-Marie-Tooth Disease**  
**Oranee Sanmaneechai, MD**, Faculty of Medicine, Siriraj Hospital, Mahidol University, Bangkok, Thailand
- 18.25 **Implications of Disease Progression During Childhood and Adolescence on Walking Speed in Charcot-Marie-Tooth Disease**  
**Sylvia Ounpuu, MSc**, Connecticut Children's Medical Center, University of Connecticut School of Medicine, Farmington, CT, USA

### INTERNATIONAL DIABETES

#### NEUROPATHY CONSORTIUM (IDNC) LEVANTE/PONENTE

**Chairs: Eva Feldman, MD, PhD**, Professor of Neurology, University of Michigan, Ann Arbor, MI, USA; **James Russell, MD, PhD**, Professor of Neurology, University of Maryland, MD, USA

- 14.30 **Risk Factors for Presence and Progression of CAN in Type 2 Diabetes**  
**Signe Toft Andersen, MD, PhD**, Aarhus University, Department of Public Health, Aarhus, Denmark
- 15.00 **Seeing What They are Feeling – The Central Nervous System in Diabetic Peripheral Neuropathy**  
**Dinesh Selvarajah, MbChB, PhD**, University of Sheffield, Sheffield, UK
- 15.30 **Deep Phenotyping to Explore Differences Between Painful and Painless Diabetic Neuropathy**  
**Andreas Themistocleous, MBBCh, PhD, FCP (SA), MRCP (UK)**, University of Oxford, Oxford, UK
- 16.00 **Sodium Channel Mutations in Small Fiber Neuropathy: Implications for Diabetic Neuropathy**  
**Daniele Cazzato, MD**, IRCCS Foundation "Carlo Besta" Neurological Institute, Milan, Italy
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

## INTERNATIONAL DIABETES

## NEUROPATHY CONSORTIUM (IDNC) Continued LEVANTE/PONENTE

**Chairs: Roy Freeman, MD**, Professor of Neurology, Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA; **David Bennett, MB PhD, FRCP**, Professor of Neurology and Neurobiology, Nuffield Department of Clinical Neurosciences, Oxford, UK

## 17.00 Oral Poster Session

- 17.00 **Sensory Neuron-derived IGF-1 Augments Neurite Outgrowth And This Autocrine/paracrine Pathway Is Suppressed in Diabetes**  
**Reza Aghanoori**, University of Manitoba, Winnipeg, Canada
- 17.05 **Deletion of SARM1 has a Protective Effect for High-fat Diet-induced Peripheral Neuropathy and Glucose Intolerance**  
**Ahmet Hoke, MD, PhD**, Johns Hopkins University, Baltimore, MD, USA
- 17.10 **Saturated and Monounsaturated Fatty Acids Differentially Regulate Nerve Function in Murine Models of Obesity**  
**Amy Rumora, PhD**, University of Michigan, Ann Arbor, MI, USA
- 17.15 **Distal Symmetric Polyneuropathy is Likely the First Neurologic Complication of Obesity**  
**Ericka Chant, MPH**, University of Michigan, Ann Arbor, MI, USA
- 17.20 **Evidence of Altered Peripheral Nerve Function in a Rodent Model of Pre-diabetes**  
**Jakir Hossain, MD**, School of Medical Sciences, UNSW, Sydney, Australia
- 17.25 **Distribution of Obesity is a Key Differentiator of Neuropathy Status**  
**Brian Callaghan, MD, MS**, University of Michigan, Ann Arbor, MI, USA
- 17.30 **Plasma Deoxydihydroceramides are Elevated in People with Diabetic Neuropathy and Correlate with Neuropathy Severity**  
**Vera Fridman, MD**, University of Colorado Anschutz Medical Campus Department of Neurology, Aurora, CO, USA
- 17.35 **Early Parallel Progression Of Peripheral And Cardiac Autonomic Nerve Dysfunction In Recent-Onset Type 1 Diabetes**  
**Gidon Bönhof, MD**, German Diabetes Center, Division of Endocrinology and Diabetology at HHU, Düsseldorf, Germany
- 17.40 **Oxidative Stress and Human Diabetic Neuropathy: Role of NADPH Oxidase 5**  
**Faye Mendelson**, University of Michigan, Ann Arbor, MI, USA

## PROGRAM – SUNDAY 23 JUNE 2019

17:45 **Invited Lecture: Emerging Concepts in the Early Detection and Management of Diabetic Neuropathy**  
**Dan Ziegler, MD**, German Diabetes Center, Leibniz Center for Diabetes Research at Heinrich Heine University, Düsseldorf, Germany

19.00-20.00 Sponsored Symposia

**CSL Behring**  
Biotherapies for Life™

### **CSL Behring Symposium: Navigating Diagnosis and Treatment Challenges in CIDP (Chronic inflammatory demyelinating polyneuropathy)** MAESTRALE

- 19.00 **Welcome and Introduction**  
**David Cornblath, MD**, Johns Hopkins University, Baltimore, MD, USA
- 19.05 **Impacts of Over and Under Diagnosis of CIDP**  
**Jeffrey Allen, MD**, University of Minnesota, Minneapolis, MN, USA
- 19.20 **Finding the Best Treatment Regimen in CIDP**  
**Pieter van Doorn, MD, PhD**, Erasmus MC University, Rotterdam, Netherlands
- 19.35 **Optimising CIDP Guidelines**  
**Peter Van den Bergh, MD, PhD**, University Hospitals St-Luc, Brussels, Belgium
- 19.50 **Q&A**  
**David Cornblath, MD**, Johns Hopkins University, Baltimore, MD, USA

**Alnylam**  
PHARMACEUTICALS  
CHALLENGE ACCEPTED

### **Alnylam Symposium: Untangling the Mixed Symptoms: Identifying and Treating hATTR Amyloidosis** GRECALE

**Chair: Laura Obici, MD**, Amyloidosis Research and Treatment Center, IRCCS, Fondazione Policlinico San Matteo, Pavia, Italy

- 19.00 **Welcome and Introductions**  
**Laura Obici, MD**, Amyloidosis Research and Treatment Center, IRCCS, Fondazione Policlinico San Matteo, Pavia, Italy
- 19.05 **hATTR Amyloidosis: Tracing the Threads of a Multisystem Disease**  
**Isabel Conceição, MD**, CHULN-Hospital Santa Maria, IMM, FML-Universidade de Lisboa, Lisboa, Portugal
- 19.20 **Treat Today or Risk Tomorrow**  
**Davide Pareyson, MD**, Fondazione IRCCS Istituto Neurologico Carlo Besta, Milan, Italy

SUNDAY

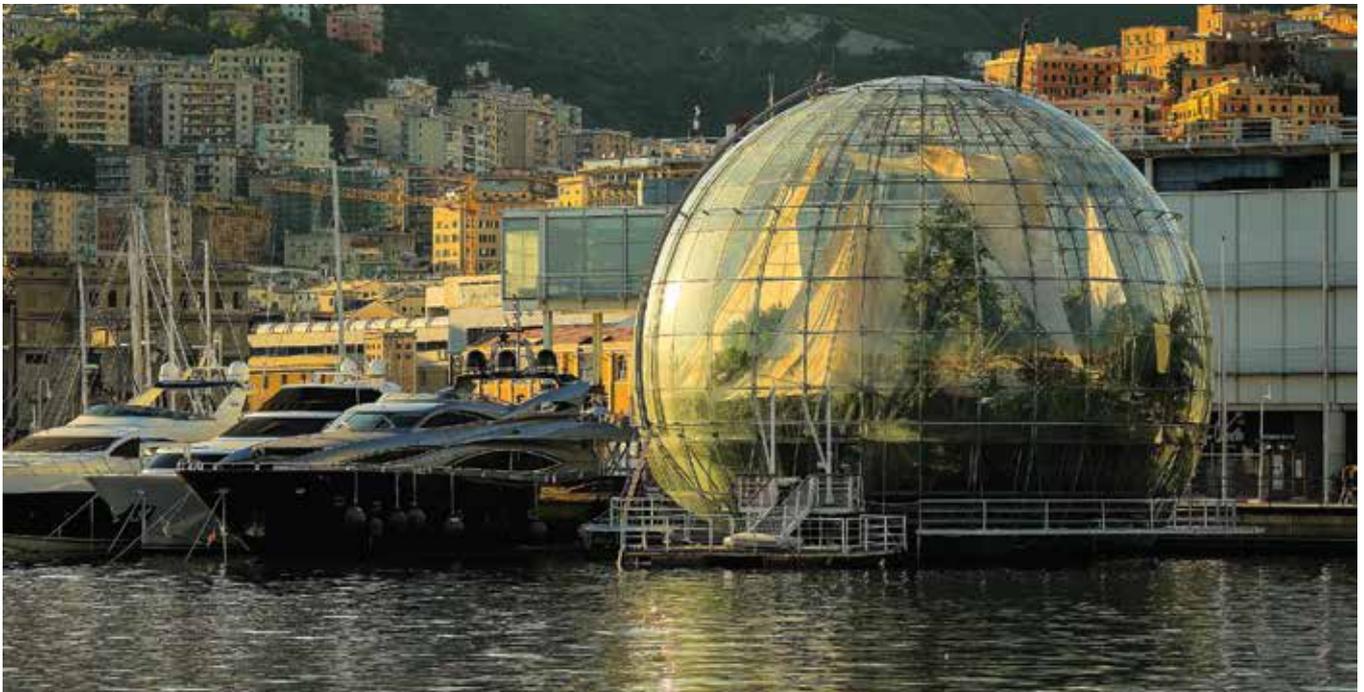
## PROGRAM – SUNDAY 23 JUNE 2019

- 19.35 **Patisiran: An RNAi Therapeutic for the Treatment of the Polyneuropathy of hATTR Amyloidosis**  
**Laura Obici, MD**, Amyloidosis Research and Treatment Center, IRCCS, Fondazione Policlinico San Matteo, Pavia, Italy
- 19.50 **Q&A**  
**Laura Obici, MD**, Amyloidosis Research and Treatment Center, IRCCS, Fondazione Policlinico San Matteo, Pavia, Italy

20.10-23.00

**Junior Reception** AQUARIUM

Sponsored by:



All are invited, as we honor Junior and new members of the Society with a reception at the Aquarium of Genoa. The aquarium is conveniently located next to the Convention Centre. Sponsored by Akcea, this reception will feature guided exhibits, food, and beverages.

Address: Ponte Spinola, 16128 Genova GE, Italy

## PROGRAM – MONDAY 24 JUNE 2019

- 7.15-8.15 Coffee & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 7.15-8.15 Poster Viewing **MODULE 7 2ND FLOOR**
- 7.15-8.15 Sponsored Symposia



### **Takeda Symposium: Thirty Years of MMN Diagnosis and Treatment: What Does the Future Hold for our Patients?** AUDITORIUM

- 7.15 **Welcome and Introductions**  
**Michel Magistris, MD**, Geneva University Hospital, Geneva, Switzerland
- 7.20 **Looking Back: Our Journey with MMN**  
**Michel Magistris, MD**, Geneva University Hospital, Geneva, Switzerland
- 7.35 **Current Diagnostic Challenges: How Can We Improve Going Forward?**  
**Eduardo Nobile Orazio, MD, PhD**, Milan University, ICRCSS Humanitas Clinical Institute, Milan, Italy
- 7.50 **Looking to the Future: Advancing the Management of MMN**  
**Leonard H. van den Berg, MD, PhD**, University Medical Center Utrecht, Utrecht, Netherlands
- 8.05 **Q&A and Panel Discussion**  
**Michel Magistris, MD**, Geneva University Hospital, Geneva, Switzerland; **Eduardo Nobile Orazio, MD, PhD**, Milan University, ICRCSS Humanitas Clinical Institute, Milan, Italy; **Leonard H. van den Berg, MD, PhD**, University Medical Center Utrecht, Utrecht, Netherlands



### **Pharnext Symposium: Charcot-Marie-Tooth Disease: New Perspectives for Patients** LEVANTE/PONENTE

- 7.15 **Welcome and Introduction**  
**David Cornblath, MD**, Johns Hopkins University, Baltimore, MD, USA
- 7.20 **CMT1A Morbidity, Evaluation and Current Management**  
**Mario Saporta, MD**, University of Miami, Miami, FL, USA
- 7.35 **CMT1A: New Translational Scientific Perspectives**  
**Michael Sereda, MD**, Max - Planck Institute for Experimental Medicine, Göttingen, Germany
- 7.50 **CMT1A: Pipeline and Results of PXT3003 International Phase III Study**  
**Peter Young, MD**, Institute for Sleep Medicine and Neuromuscular Disorders, Münster, Germany
- 8.05 **Conclusions – Q/As – Closing Remarks**  
**David Cornblath, MD**, Johns Hopkins University, Baltimore, MD, USA

MONDAY

## PROGRAM – MONDAY 24 JUNE 2019

8.30-9.00

### Arthur K Asbury Lecture: Skin for Neurologists AUDITORIUM

**Giuseppe Lauria, MD, IRCCS "Carlo Besta" Neurological Institute & University of Milan, Milan, Italy**

**Chair: Eduardo Nobile Orazio, MD, PhD, Milan University, IRCCS Humanitas Clinical Institute, Milan, Italy**



Giuseppe Lauria is Professor of Neurology and director of the Department of Clinical Neurosciences at the IRCCS "Carlo Besta" Neurological Institute of Milan, Italy. He received the degree in Medicine at the University of Padova and trained as a resident in Neurology at the University of Ferrara, and was a fellow at the Department of Neuroscience at Johns Hopkins University, USA. He has pioneered the use of skin biopsy in peripheral neuropathies and neuropathic pain syndromes, contributed to the discovery of new phenotypes and gene mutations in small fibre neuropathies and participated in setting guidelines for clinical treatment of peripheral neuropathies.

9.00-10.30

### Platform Session II AUDITORIUM

**Chairs: Nortina Shahrizaila, PhD, University of Malaya, Kuala Lumpur, Malaysia; Douglas Zochodne, MD, FRCPC, University of Alberta, Edmonton, Canada**

- 9.00 **Analysis of 193 Whole Genome Sequencing Data to Understand Neuropathic Pain Disorders**  
**Andreas Themistocleous, MBBCh, PhD, University of Oxford, Oxford, UK**
- 9.15 **A Recessive Repeat Expansion Causes CANVAS and is a Common Cause of Late-Onset Sensory Ataxia**  
**Andrea Cortese, MD, PhD, Department of Neuromuscular Disease, UCL Institute of Neurology, London, UK**
- 9.30 **Whole Genome Sequencing in CMT Cases from the 100,000 Genome Project**  
**Menelaos Pipis, MBBS, MRC Centre for Neuromuscular Diseases, UCL Queen Square Institute of Neurology, London, UK**
- 9.45 **Depleted Systemic Markers of Neuroinflammation And Growth Factors In Type 2 Diabetes Patients With Polyneuropathy**  
**Gidon Bönhof, MD, German Diabetes Center, Division of Endocrinology and Diabetology at HHU, Düsseldorf, Germany**
- 10.00 **Protective Effects of Endogenously Expressed Calpain Inhibitor in a Mouse Model of Guillain-Barré Syndrome**  
**Rhona McGonigal, MSci, PhD, University of Glasgow, Glasgow, UK**
- 10.15 **Optimizing Electrodiagnosis for Chronic Inflammatory Demyelinating Polyneuropathy with Automated Analysis and Machine Learning**  
**Ilse Lucke, MD, Amsterdam UMC - University of Amsterdam, Amsterdam, Netherlands**

10.30-11.00

Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

MONDAY

## PROGRAM – MONDAY 24 JUNE 2019

11.00-11.30

### Jack Griffin Lecture: Neuronal and Glial Mechanisms Regulating Nerve Regeneration AUDITORIUM

**Valeria Cavalli, PhD**, *Washington University in St Louis, School of Medicine, Saint Louis, MO, USA*

**Chair: Robert Baloh, MD, PhD**, *Cedars-Sinai Medical Center, Los Angeles, CA, USA*



Dr. Cavalli's work aims to understand the complex cascade of cellular events responsible for repairing damaged axons in peripheral nerves and to identify therapeutic targets to improve neuronal recovery following axon injury. Dr. Cavalli and her colleagues have identified several key molecular players and their roles in announcing injury, initiating a response and carrying out repair. Among them are mammalian Target of Rapamycin (mTOR), JNK-interacting protein, JIP3 and the gene regulators HDAC5 and HIF-1alpha. Dr. Cavalli earned her bachelor's, master's and doctoral degrees from the University of Geneva, Switzerland. During her graduate work in the lab of Dr. Jean Gruenberg in Geneva, Dr. Cavalli studied the signaling mechanisms regulating membrane trafficking in cells. She continued with postdoctoral training at the University of California, San Diego. There she joined the lab of Dr. Larry Goldstein and studied how vesicular transport impacts signaling along peripheral nerves, and, vice versa, how signaling impacts vesicular transport. In her initial studies, she focused on retrograde injury signaling, or how information about an injury is conveyed from the distantly located lesion site in the axon back to the cell body. She received a post-doctoral fellowship from the Christopher and Dana Reeve Foundation to continue her studies on injury signaling. She then joined the Washington University faculty in 2006 and is now Associate Professor in the Department of Neuroscience.

11.30-12.30

### Oral Poster Session II AUDITORIUM

**Chairs: Ruth Stassart, MD**, *University of Leipzig, Leipzig, Germany;*

**Chiara Briani, MD**, *University of Padova, Padova, Italy*

- 11.30 **Acute Small Fibre Neuropathy: A Neglected Condition?**  
**Thierry Gendre, MD**, *Department of Neurology, Hôpital Henri Mondor, AP-HP, Université Paris-Est, Créteil, France*
- 11.35 **Ultrastructural Mechanisms of Macrophage-induced Demyelination in Guillain-Barré Syndrome**  
**Haruki Koike**, *Nagoya University Graduate School of Medicine, Nagoya, Japan*
- 11.40 **Antibody- and Macrophage-mediated Internodal Demyelination in CIDP: Clinical, Electrophysiological, Immunological and Pathological Correlations**  
**Jean-Michel Vallat, MD**, *National Reference Center for Rare Peripheral Neuropathies and Department of Neurology, Limoges, France*
- 11.45 **Prognostic Features for Death and Progression in Patients with POEMS Syndrome**  
**Stephen Keddle**, *MRC Centre for Neuromuscular Diseases National Hospital for Neurology and Neurosurgery, London, UK*

MONDAY

## PROGRAM – MONDAY 24 JUNE 2019

- 11.50 **Consequences of SAC3/FIG4 Deficiency to Phosphoinositides in Fibroblasts of Patients with CMT4J**  
**Jun Li, MD, PhD**, Wayne State University School of Medicine, Detroit, MI, USA
- 11.55 **Adult Polyglucosan Body Disease Presenting with a Peripheral Neuropathy: Broadening the Clinical Spectrum**  
**Jonathan De Winter**, Antwerp University Hospital, Edegem, Belgium
- 12.00 **Schwann Cell Transcript Biomarkers for Hereditary Neuropathy Skin Biopsies**  
**John Svaren, PhD**, University of Wisconsin, Madison, WI, USA
- 12.05 **Unravelling Hallmarks of Axonal Degeneration in Charcot-Marie-Tooth Type 2 Using Induced Pluripotent Stem Cells**  
**Jonas Van Lent**, University of Antwerp, Antwerp, Belgium
- 12.10 **Altered Nerve Triglycerides in Mouse Models of Diabetes with Neuropathy**  
**Phillipe O'Brien, PhD**, University of Michigan Medical School, Ann Arbor, MI, USA
- 12.15 **Risk Factors for the Development of Chemotherapy Induced Peripheral Neuropathy: A Retrospective Study**  
**Noah Kolb, MD**, Robert Larner College at Medicine at the University of Vermont, Burlington, VT, USA

12.30-13.00 **PNS Annual General Meeting** (all are welcome) **AUDITORIUM**

13.00-14.30 Lunch & Exhibits **MODULE 8 & 7 1ST FLOOR**

13.00-14.30 Poster Session II **MODULE 7 2ND FLOOR**

14.30-18.30 SPECIAL INTEREST GROUP (SIG) PARALLEL SESSIONS

### **INFLAMMATORY NEUROPATHY CONSORTIUM (INC)** MAESTRALE

- 14.30 **INC Project Updates**  
**Peter Van den Bergh, MD**, Université Catholique de Louvain and Director of the Neuromuscular Centre of Reference of the Cliniques Universitaires Saint-Luc, Brussels, Belgium
- 14.30 **Grant Opportunities Available through the GBS | CIDP Foundation**  
**Lisa Butler**, Executive Director of GBS/CIDP Foundation
- 14.32 **Update on IGOS**  
**Bart Jacobs, MD, PhD**, Erasmus MC University Medical Center, Rotterdam, Netherlands
- 14.40 **Update on Italian CIDP Databases**  
**Eduardo Nobile-Orazio, MD, PhD**, Milan University, Milan, Italy
- 14.50 **Update on IMAGiNE Study**  
**Marielle Prüppers, MD**, Maastricht University Medical Center, Maastricht, Netherlands

- 15.00 **Ultrasound and MRI of Peripheral Nervous System: Usefulness for Diagnosis and Therapeutic Indication**  
**Luca Padua, MD, PhD**, *Università Cattolica del Sacro Cuore - Fondazione Policlinico Universitario, A. Gemelli, IRCCS, Rome, Italy*;  
**Simonetta Gerevini, MD**, *IRCCS San Raffaele Hospital, Milano, Italy*  
**Chair: Stephan Goedee, MD**, *UMC Utrecht, Utrecht, Netherlands*
- 16.00 **Oral Abstracts**  
**Chairs: Emilien Delmont, MD**, *Hôpital La Timone Adulte, Marseille, France*; **Luis Querol, MD, PhD**, *Hospital de la Santa Creu I Sant Pau, Barcelona, Spain*
- 16.00 **Outcomes After Single-Cycle Rituximab in Patients with Anti-MAG Polyneuropathy: An Average Eleven Years Follow-up Analysis**  
**Martina Garnero**, *Neurology Department, Ospedale Sanremo, ASL 1, Sarema, Italy*
- 16.10 **One Year Closer to Clinical Trials with a New Antigen Specific Treatment for Anti-MAG Neuropathy**  
**Pascal Hänggi**, *University of Basel, Basel, Switzerland*
- 16.20 **POEMS Syndrome: Characterization of Neuropathy and Post-Treatment Outcome in 36 Patients**  
**Nathalie Deschamps**, *CHU Bicetre, CHU Pointe à Pitre, Kremlin Bicetre, France*
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Neuropathology of Inflammatory Neuropathies**  
**Haruki Koike, MD, PhD**, *Nagoya University Graduate School of Medicine, Nagoya, Japan*  
**Chair: Judith Spies, PhD**, *Royal Prince Alfred Hospital, Camperdown, Australia*
- 17.30 **Oral Abstracts**  
**Chairs: Hugh Willison, MD**, *University of Glasgow, Glasgow, UK*;  
**Umapathi Thirugnanam, MD**, *National Neuroscience Institute, Singapore, Singapore*
- 17.30 **Misdiagnosis and Diagnostic Pitfalls of CIDP**  
**Merel Broers**, *Department of Neurology, Erasmus MC, University Medical Center Rotterdam, Netherlands, Rotterdam, Netherlands*
- 17.40 **Immunomodulatory Effects of Bortezomib in Experimental Autoimmune Neuritis in Lewis Rats**  
**Rafael Klimas**, *Ruhr-University Bochum, St. Joseph University Hospital Bochum, Bochum, Germany*

## PROGRAM – MONDAY 24 JUNE 2019

MONDAY

- 17.50 **Difference of Clinical and Paraclinical Patterns in anti-FGFR 3-Positive Sensory Neuropathy Cases from Brazil and Europe**  
**Yannick Tholance, PharmD, PhD**, *Synaptopathies and Autoantibodies (INMG), Department of Biochemistry in University Hospital of Saint-Etienne, Saint-Etienne, France*
- 18.00 **Antibodies Directed Against Peripheral Neurons, Schwann Cells and Myelin are Frequently Found in Zika-exposed Subjects**  
**Simon Rinaldi, MBChB, PhD**, *University of Oxford, Oxford, UK*
- 18.10 **Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) Associated with Sarcoidosis or Connective Tissue Disease**  
**Clément Vialatte de Pémille**, *Service de neurologie CHU de Bicêtre Paris, Service de neurologie Hôpital Saint Joseph Paris, Paris, France*
- 18.20 **Holistic Characterization of the Repertoire of Targeted Autoantigens of Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)**  
**Christian Moritz, PhD**, *Institut NeuroMyoGene INSERM U1217/ CNRS UMR 5310 at the University of Lyon, Saint-Étienne, France*

### CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM (CMTR) GRECALE

- 14.30 **Invited Lecture: Rare and Genetically Undiagnosed Forms of Inherited Peripheral Neuropathies**  
**Jan Senderek, MD**, *Friedrich-Baur-Institute at the Department of Neurology, LMU Munich, Munich, Germany*  
**Chair: Gian Maria Fabrizi, MD, PhD**, *University of Verona, Verona, Italy*
- 15.00 **Platform Presentations: Genetics, Biomarkers, Amyloidosis**  
**Chairs: Teresa Coelho, MD**, *Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal*; **Andrea Cortese, MD, PhD**, *UCL Institute of Neurology, London, UK*
- 15.00 **Broadening the Spectrum of Biallelic ADPRHL2 Mutations into Complex Early-Onset Motor Neuropathy Phenotypes**  
**Danique Beijer**, *University of Antwerp, Antwerp, Belgium*
- 15.15 **Deep-Learning Based Morphological Profiling for Rapid Variant Annotation in Inherited Neuropathies**  
**Wolfgang Pernice, PhD**, *Columbia University Medical School, New York, NY, USA*
- 15.30 **Long-term Safety and Efficacy of Patisiran in Patients with hATTR Amyloidosis: Global OLE Study**  
**Michael Polydefkis, MD, MHS**, *Johns Hopkins University, Baltimore, MD, USA*

## PROGRAM – MONDAY 24 JUNE 2019

- 15.45 **Wildtype and Familial Transthyretin Amyloid Polyneuropathy: Distinct Cutaneous Biomarkers at the Distal Limb**  
**Gigi Ebenezer, MBBS, MD**, Johns Hopkins School of Medicine, Baltimore, MD, USA
- 16.00 **Plasma NfL Concentration is Increased in Patients with ATTRm and Correlates with Clinical Severity Scores**  
**Mahima Kapoor**, MRC Centre for Neuromuscular Diseases, UCL Queen Square Institute of Neurology, London, UK
- 16.15 **Identification of TMPRSS5/Spinesin, a Novel Schwann Cell Derived Plasma Biomarker for CMT1A**  
**Matthew Davison**, Sanofi, Framingham, MA, USA
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 17.00 **Measuring Disability of Infants, Children and Adults with CMT**  
**Joshua Burns, PhD**, The University of Sydney, Sydney, Australia  
**Chair: Michael Shy, MD**, University of Iowa, IA, USA
- 17.30 **Platform Presentations: Therapy**  
**Chairs: Wilson Marques Jr, MD**, School of Medicine of Ribeirão Preto, Ribeirão Preto, Brazil and **Chiara Pisciotta, MD, PhD**, University of Iowa, IA, USA
- 17.30 **Preclinical Gene Therapy Studies for FIG4/CMT 4J and GARS/CMT 2D**  
**Robert Burgess, PhD**, The Jackson Laboratory, Bar Harbor, ME, USA
- 17.45 **Treatment of Arg98Cys MPZ Mice In Vitro and In Vivo with IFB088**  
**Michael Shy, MD**, University of Iowa, Carver College of Medicine, Iowa City, IA, USA
- 18.00 **IFB-088 Treatment Improves Charcot-Marie-Tooth Type 1A Disease Phenotype of C3-PMP22 Mice**  
**Maurizio D'Antonio, PhD**, IRCCS Ospedale San Raffaele, Milan, Italy
- 18.15 **Oral Posters: Therapy**
- 18.15 **Novel Biomarkers and Therapeutic Approaches in Charcot-Marie-Tooth Disease (CMT)**  
**Michael Sereda, MD**, University Medical Centre Goettingen, Max-Planck-Institute of Experimental Medicine, Goettingen, Germany
- 18.20 **Longitudinal Assessment of Iowa FAP Cohort Treated with siRNA**  
**Shawna Feely, MS, CGC**, University of Iowa, Iowa City, IA, USA

- 18.25 **Patisiran, A Silencing RNA in Hereditary Transthyretin Amyloid Polyneuropathy: First Experience in Real Life**  
**Thierry Gendre, MD**, Department of Neurology, Henri Mondor Hospital, East Paris University, Créteil, France

**INTERNATIONAL DIABETES NEUROPATHY CONSORTIUM (IDNC) LEVANTE/PONENTE**

**Chairs: Rayaz Malik, MB ChB, PhD**, Professor of Medicine Weill Cornell Medicine, Qatar, Doha, Qatar; **Rodica Pop Busui, MD, PhD** Professor of Medicine, University of Michigan, Ann Arbor, MI, USA

- 14.30 **Ultrasound of Peripheral Nerve in Diabetes**  
**Stefan Meng, MD**, KFJ Hospital, Vienna, Austria
- 15.00 **MR (Magnetic resonance) Neurography in Peripheral Neuropathies**  
**Jennifer Kollmer, MD**, Heidelberg University Hospital, Department of Neuroradiology, Heidelberg, Germany
- 15.30 **Testing Autonomic Outcomes in Diabetic Neuropathy Research: Challenges and Advantages**  
**James Russell, MD, MS**, University of Maryland, Baltimore, MD, USA
- 16.00 **Scientific Pitfalls in Emerging Technology for Study of Neuropathy**  
**Brian Callaghan, MD, MS**, University of Michigan, Ann Arbor, MI, USA  
**Christopher Gibbons, MD**, Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA
- 16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

**INTERNATIONAL DIABETES NEUROPATHY CONSORTIUM (IDNC) Continued LEVANTE/PONENTE**

**Chairs: Doug Wright, PhD**, Kansas University Medical Center, Kansas City, KS, USA; **Christopher Gibbons, MD, MMSc**, Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA

- 17.00 **Oral Poster Session**
- 17.00 **Tale of Two States: A Comparison Study of Baseline Health Data of Neuropathy Patients**  
**Mamatha Pasnoor, MD**, the University of Kansas Medical Center, Kansas City, KS, USA
- 17.05 **Expression of GAP-43 in Type 2 Diabetes and IGT: A Longitudinal Study**  
**Xin Pan, MD**, Johns Hopkins School of Medicine, Baltimore, MD, USA

## PROGRAM – MONDAY 24 JUNE 2019

- 17.10 **Genome-wide DNA Methylation Profiling Identifies Epigenetic Clues into Human Peripheral Neuropathy in Type 2 Diabetes**  
**Stephanie Eid, MD**, *University of Michigan, Ann Arbor, MI, USA*
- 17.15 **Intraepidermal Nerve Fiber Regeneration is Different Between Type1 and Type2 Diabetes**  
**Mohammad Khoshnoodi, MD**, *Johns Hopkins University, Baltimore, MD, USA*
- 17.20 **Epidermal Axon Changes in Patients with Prediabetes: The PACMAN Study**  
**Dan Elliott, BS**, *University of Kansas Medical Center, Kansas City, KS, USA*
- 17.30 **Platform Session**
- 17.30 **Gut Microbiome and its Potential Role in Obesity-induced Allodynia**  
**Raiza Bonomo**, *Loyola University Chicago, Chicago, IL, USA*
- 17.45 **A Keratinocyte-Derived Mechanism of Nicotinamide Riboside to Prevent and Reverse Diabetic Neuropathy**  
**Cheng-Ying Ho, MD, PhD**, *University of Maryland School of Medicine, Baltimore, MD, USA*
- 18.00 **Mrgprd as a Potential Therapeutic Target for Painful Diabetic Neuropathy**  
**Daniela Menichella, MD, PhD**, *Northwestern University, Chicago, IL, USA*
- 18.15 **Real Time Analysis of ATP Levels in DRG Neurons Derived from Normal or Diabetic Rats**  
**Reza Aghanoori**, *University of Manitoba, Winnipeg, Canada*
- TOXIC NEUROPATHY CONSORTIUM (TNC) LIBECCIO**  
**Chairs: Sandra Reiger, PhD**, *University of Miami, Coral Gables, FL, USA*;  
**Daniela Salvemini, PhD**, *King's College London, University of London, London, UK*
- 14.30 **Summary/Update of Activities**  
**Guido Cavaletti, MD**, *University of Milano-Bicocca, Monza (I), Milan, Italy*
- 15.00 **The Power of Neurophysiology in Chemotherapy-induced Peripheral Neuropathy**  
**Susanna Park, PhD**, *University of Sydney, Sydney, Australia*

## PROGRAM – MONDAY 24 JUNE 2019

### 15.30 Oral Abstracts

- 15.30 **Outcome Measures in the Assessment of Chemotherapy Induced Peripheral Neuropathy – Which Tools are Most Responsive?**  
**Tiffany Li, MBIostat BSc, University of Sydney, Sydney, Australia**
- 15.40 **Bortezomib Neuropathy: Clinical and Electrophysiological Features and its Predictive Factor**  
**Nagaaki Katoh, Shinshu University, Matsumoto, Japan**
- 15.50 **Incidence and Characteristics of Neurological Adverse Events Secondary to Immunotherapy with Checkpoint Inhibitors**  
**Roser Velasco, Unit of Neuro-Oncology, Hospital Universitari de Bellvitge-ICO L'Hospitalet-IDIBELL, Barcelona, Spain**
- 16.00 **Elevated Neurofilament Light Chain (NF-L) Levels in Pancreatic Cancer Patients with CIPN Receiving Abraxane**  
**Catherine Stehman-Breen, MD, Disarm Therapeutics, Cambridge, MA, USA**
- 16.10 **Asymmetry in Chemotherapy-induced Peripheral Neuropathy: Differences in Patient Report and Objective Assessment**  
**Hannah Timmins, BSc (Hons), University of Sydney, Sydney, Australia**

16.30 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

### **TOXIC NEUROPATHY CONSORTIUM (TNC) Continued** LIBECCIO

**Chairs: Andreas Argyriou, MD, PhD, General Hospital of Patras, Patras, Greece;**  
**Nathan Staff, MD, PhD, Mayo Clinic, Rochester, MN, USA**

### 17.00 Oral Abstracts

- 17.00 **Mitochondrial Vacuolation Occurs Independent of Axon Degeneration in Paclitaxel-induced Peripheral Neuropathy**  
**Anthony Cirrincione, BSc, University of Miami Department of Biology, Coral Gables, FL, USA**
- 17.10 **Molsidomine Provides Neuroprotection Against Vincristine-induced Peripheral Neurotoxicity**  
**Francesco Lotti, Department of Pathology & Cell Biology, Columbia University Medical Center, New York, NY, USA**
- 17.20 **Effects of Candesartan on Mouse Models of Vincristine- and Oxaliplatin-induced Neuropathy**  
**Hichem Bouchenaki, EA 6309 - Myelin Maintenance & Peripheral Neuropathy, Faculties of Medicine and Pharmacy, University of Limoges, Limoges, France**

## PROGRAM – MONDAY 24 JUNE 2019

17.30 **Relevance of Peripheral Neuropathy in Cancer Patients Treated with Chemotherapy**  
**Alberto Sobrero, MD**, Ospedale San Martino, Genova, Italy

18:00 **Invited Lecture: Palliative Care in Oncology and Neuropathic Pain**  
**Augusto Caraceni, MD**, Fondazione IRCCS Istituto Nazionale dei Tumori di Milano, Milano, Italy

19.00-20.00 Sponsored Symposia

### **GRIFOLS** Grifols Symposium: Improving IVIG Dosing in Autoimmune Neuropathies MAESTRALE

19.00 **SPIN Award Ceremony**

19.10 **IVIG Dosing in Guillain-Barre Syndrome (GBS)**  
**Bart Jacobs, MD, PhD**, Erasmus MC, University Medical Center Rotterdam, Rotterdam, Netherlands

19.25 **IVIG Dosing in Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)**  
**John England, MD, FAAN**, LSU Healthcare Network Clinic, New Orleans, LA, USA

19.40 **IVIG Dosing in Multifocal Motor Neuropathy (MMN)**  
**JM. Léger, MD**, National Referral Center for Rare Neuromuscular Diseases, University Hospital Pitié-Salpêtrière, Paris, France



### **Akcea Symposium: A '3D Look' at hATTR Amyloidosis: Diagnosis, Disease Progression and Defining Real World Treatment Outcomes** GRECALE

**Chair: Teresa Coelho, MD**, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal

19.00 **Welcome**  
**Teresa Coelho, MD**, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal

19.05 **hATTR Amyloidosis: How Do We Rapidly Diagnose the Disease and Evaluate Polyneuropathy Impairment?**  
**Chris Klein, MD**, Mayo Clinic, Rochester, MN, USA

19.15 **How Do We Define Disease Progression?**  
**Laura Obici, MD**, University of Pavia, Pavia, Italy

19.25 **Can We Predict Treatment Failure?**  
**Teresa Coelho, MD**, Hospital de Santo António - Centro Hospitalar do Porto, Porto, Portugal

19.35 **Debate: How Do We Assess Disease Progression in the Real World?**

MONDAY

## PROGRAM – TUESDAY 25 JUNE 2019

- 7.15-8.15 Coffee & Exhibits **MODULE 8 & 7 1ST FLOOR**
- 7.15-8.15 Poster Viewing **MODULE 7 2ND FLOOR**
- 7.15-8.15 Sponsored Symposia



### LFB Symposium: Evidence-based Medicine and Current Advances in Multifocal Motor Neuropathy **AUDITORIUM**

**Chairs:** **Jean-Marc Léger, MD**, Center for Rare Neuromuscular Diseases, Centre Hospitalo-Universitaire Pitié-Salpêtrière (or Pitié-Salpêtrière University Hospital) Paris, France; **Hans Peter Hartung, PhD**, Universität Dusseldorf, Dusseldorf, Germany

- 7.15 **Epidemiology, Pathophysiology and New Perspectives in Multifocal Motor Neuropathy: An Update**  
**Hans Peter Hartung, PhD**, Universität Dusseldorf, Dusseldorf, Germany
- 7.30 **Multifocal Motor Neuropathy: Diagnosis and Treatment Monitoring**  
**Yusuf Rajabally, MD, PhD**, Queen Elizabeth Hospital, Birmingham, UK
- 7.45 **A Randomized Comparative Trial with 2 IVIGs intravenous immunoglobulins in Multifocal Motor Neuropathy: The LIME Study Results**  
**Jean-Marc Léger, MD**, Center for Rare Neuromuscular Diseases, Centre Hospitalo-Universitaire Pitié-Salpêtrière (or Pitié-Salpêtrière University Hospital) Paris, France
- 8.00 **Wrap-Up**

### 8.30-9.00 **PK Thomas Lecture: Familial Amyloid Polyneuropathy as a Model of Medical Progress** **AUDITORIUM**

**David Adams, MD, PhD, APHP**, Univ Paris Sud, Le Kremlin Bicetre, France  
**Chair:** **Davide Pareyson, MD**, Carlo Besta Neurologic Institute, Milan, Italy



David Adams is Head of the French National Reference Centre for FAP, a position held since 2005, and has been the Coordinator of the French Network for FAP (CORNAMYL) since 2010. He was made a Professor of Neurology at the Université Paris Sud, France in 1996, and between 1996 and 2008 was Assistant Professor of the Department of Neurology at the Assistance Publique-Hôpitaux de Paris (AP-HP), Centre Hospitalier Universitaire Bicêtre, Université Paris Sud. In 2009, he became Head of the Department of Neurology at AP-HP. He is cluster Manager of Neurosciences Head and Neck since December 2016. He is now involved in research programmes of early diagnosis in sporadic cases and genetic carriers, and belongs to the European Reference Network for neuromuscular diseases ERN EURO NMD. His main areas of expertise are peripheral neuropathies, including FAP and other rare peripheral neuropathies. He has been particularly involved in the evaluation of the effects of liver transplantation on neuropathy in FAP patients in France since 1993, and the neurological risks of domino liver transplantation. He is Principal Investigator in many multicentric clinical trials for FAP. Professor Adams was an Advisory Board member for International Symposium on Familial Amyloidotic Polyneuropathy (ISFAP), the International Symposium on Liver Transplantation in Familial Amyloidotic Polyneuropathy (IS-LTFAP). He organized the First European Congress on Hereditary ATTR amyloidosis in 2015 and the First European meeting for ATTR amyloidosis for doctors and patients in 2017 in Paris. He received the 2017 PK Thomas prize from the European Academy of Neurology.

## PROGRAM – TUESDAY 25 JUNE 2019

9.00-10.30

### Platform Session III AUDITORIUM

**Chairs: Satoshi Kuwabara, MD, Chiba University, Chiba, Japan; Michael Shy, MD, Carver College of Medicine – University of Iowa, Iowa City, IA, USA**

- 9.00 **The Impact of Eculizumab on Neurological Improvement in Guillain–Barré Syndrome: Subanalysis of JET-GBS Study**  
**Sonoko Misawa, Department of Neurology, Chiba University Graduate School of Medicine, Chiba, Japan**
- 9.15 **Intrinsic Growth and Plasticity Pathways Within Sensory Neurons: An Expanding List**  
**Douglas Zochodne, MD, FRCPC, University of Alberta, Edmonton, Canada**
- 9.30 **Long-Term Efficacy and Safety of Inotersen for Hereditary Transthyretin Amyloidosis: NEURO-TTR Open-Label Extension 2-Year Update**  
**Thomas Brannagan, MD, Columbia University Medical Center, New York, NY, USA**
- 9.45 **Autoantibodies and Pain: The Role of Leucine-Rich Glioma Inactivated 1 in Primary Sensory Neurons**  
**John Dawes, University of Oxford, Oxford, UK**
- 10.00 **Gene Therapy for Peripheral Neuropathy CMT1A**  
**Nicolas Tricaud, PhD, INSERM U1051, Institut des Neurosciences de Montpellier, Montpellier, France**
- 10.15 **Gene Replacement Therapy for CMT1X Neuropathy**  
**Alexia Kagiava, The Cyprus Institute of Neurology and Genetics and Cyprus School of Molecular Medicine, Nicosia, Cyprus**

10.30-11.00

Coffee Break & Exhibits MODULE 8 & 7 1ST FLOOR

11.00-11.30

### Pembroke Lecture: The Impact of Mitochondrial Biology on Central and Peripheral Nervous System Disorders AUDITORIUM

**Massimo Zeviani, MD, PhD, The Clinical School, University of Cambridge, and the MRC Mitochondrial Biology Unit, Cambridge, UK**

**Chair: Mary Reilly, MD, FRCP, FRCPI, UCL Institute of Neurology, London, UK**



Professor Massimo Zeviani is the Director of the University of Cambridge/MRC-Mitochondrial Biology Unit in Cambridge, UK. He moved to UK from Milano, Italy, where he directed the Unit of Molecular Neurogenetics at the Fondazione Istituto Neurologico Carlo Besta. By characterizing informative patients and families affected by mitochondrial impairment, Prof Zeviani identified many disease-related genes and developed the first therapeutic approaches for mitochondrial diseases. His team has created several mouse models for the study of the molecular and cellular mechanisms of mitochondrial disorders, in addition to maintaining a clinical interest in the management and investigation of mitochondrial disease patients. His research has also provided a relevant contribution to the characterization of the role of mitochondrial dysfunction in the pathogenesis of several neurodegenerative conditions, involving the central and peripheral nervous systems.

TUESDAY

## PROGRAM – TUESDAY 25 JUNE 2019

- 11.30-12.30 **Oral Poster Session III** AUDITORIUM  
**Chairs: Charlotte Sumner, MD, John Hopkins Medicine, Baltimore, MD, USA; Luana Benedetti, MD, PhD, Azienda Ospedaliera Univesitaria San Martino di Genova, Genova, Italy**
- 11.30 **Bortezomib Neurotoxicity is Associated with Altered MAP2 Levels and Distribution within Human iPSC-Derived Sensory Neurons**  
**Nathan Staff, MD, PhD, Mayo Clinic, Rochester, MN, USA**
- 11.35 **Rate of Progression of Utah Early Neuropathy Scale (UENS) Score in Diabetic Neuropathy**  
**J. Robinson Singleton, MD, University of Utah, Salt Lake City, UT, USA**
- 11.40 **Biomarker Profiling of Neuropathic Pain in Idiopathic Peripheral Neuropathy**  
**Perry Van Doormaal, MD, PhD, Johns Hopkins University School of Medicine, Baltimore, MD, USA**
- 11.45 **Influence of Body Mass Index on Disability in Children with CMT**  
**Gabrielle Donlevy, BAppSc, MNutrDiet, The University of Sydney, The Children's Hospital at Westmead, Sydney, New South Wales, Australia, Sydney, Australia**
- 11.50 **A Mitochondrial ATP6 Mutation Causing a Slowly Progressive Myeloneuropathy**  
**Tanya Bardakjian, MS, LCGC, University of Pennsylvania, Philadelphia, PA, USA**
- 11.55 **Nusinersen in Adults with Spinal Muscular Atrophy, A Single Center Experience**  
**Orly Moshe-Lilie, MD, Oregon Health and Science University (OHSU), Portland, OR, USA**
- 12.00 **The Overlapping Spectrum of Chronic Inflammatory Demyelinating Polyradiculoneuropathy and Anti-MAG Neuropathy**  
**Giuseppe Liberatore, Neuromuscular and Neuroimmunology Service, Humanitas Clinical and Research Institute – IRCCS, Rozzano, Milan, Italy**
- 12.05 **International Validation of the modified Erasmus GBS Outcome Score (mEGOS) for Guillain-Barré Syndrome**  
**Alex Doets, Erasmus MC University Medical Center Rotterdam, Rotterdam, Netherlands**
- 12.10 **Incidence of Antibodies Against the Node of Ranvier in a Prospective Cohort of 1000 CIDP**  
**Emilien Delmont, MD, PhD, Referral Centre for Neuromuscular Diseases and ALS, APHM, Marseille, France**
- 12.15 **Secondary Endpoints (PATH Extension Study): Long-term Outcomes of Subcutaneous Immunoglobulin IgPro20 in CIDP Maintenance Treatment**  
**Ivo van Schaik, University of Amsterdam, Amsterdam, Netherlands**

## PROGRAM – TUESDAY 25 JUNE 2019

12.30-14.30 Lunch & Exhibits **MODULE 8 & 7 1ST FLOOR**

12.30-14.30 Poster Session III **MODULE 7 2ND FLOOR**

14.30-16.30 **Clinical Trial Updates** **AUDITORIUM**

**Chairs: Mary Reilly, MD, FRCP, FRCPI, UCL Institute of Neurology, London, UK;  
David Cornblath, MD, Johns Hopkins University, Baltimore, MD, USA**

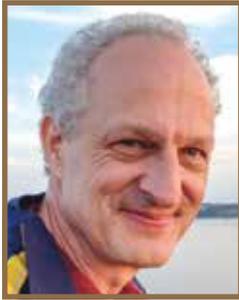
- 14.30 **Improving Neuropathy and Mobility in Diabetes: the INMED Trial**  
**Lindsay Zilliox, MD, MS, University of Maryland, VA Maryland Health Care System, Baltimore, MD, USA**
- 14.43 **Second IVIg Course in Guillain-Barré Syndrome Patients with Poor Prognosis (SID-GBS) Second IVIg Dose in Guillain-Barré Syndrome; Double-blind Randomized Controlled Trial**  
**Christa Walgaard, Erasmus Medical Center, Rotterdam, Netherlands**
- 14.56 **A Dose Response RCT Of IV Immunoglobulin in Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)/DRIP**  
**Krista Kuitwaard, MD, PhD, Erasmus MC University Medical Center, Albert Schweitzer Hospital, Rotterdam, Dordrecht, Netherlands**
- 15.09 **Intravenous Immunoglobulin Overtreatment in Chronic Inflammatory Demyelinating Polyneuropathy: Double-blind Randomized Controlled Non-Inferiority Trial (IOC-TRIAL)**  
**Max Adrichem, Amsterdam UMC - University of Amsterdam, Amsterdam, Netherlands**
- 15.22 **Restabilization after IVIg Withdrawal in Patients with Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)**  
**Ilse Lucke, MD, Amsterdam UMC - University of Amsterdam, Amsterdam, Netherlands**
- 15.35 **IVIg intravenous immunoglobulin Treatment-Related Fluctuations in CIDP Patients Using Daily Grip Strength Measurements (GRIPPER): Study Update**  
**Jeffrey Allen, University of Minnesota; Northwestern University, Minneapolis, MN, USA**
- 15.48 **A Randomized, Single-Blinded, Non-Inferiority Cross-Over Trial of Facilitated Subcutaneous Immunoglobulin in Multifocal Motor Neuropathy**  
**Ali Al-Zuhairy, Department of Neurology, Neuroscience Center, Copenhagen University Hospital (Rigshospitalet), Copenhagen, Denmark**
- 16.01 **RECIPE: A Phase II Randomized Controlled Trial of Rituximab for Refractory CIDP with IgG4 Autoantibodies**  
**Masahiro Lijima, MD, PhD, Department of Advanced Medicine, Nagoya University Hospital, Nagoya, Japan**
- 16.14 **Efficacy and Safety of PXT3003 in Patients with CMT1A: International Pivotal Phase III Trial**  
**Shahram Attarian, MD, PHD, AP-HM et Aix Marseille Université, Marseille, France, Marseille, France**

TUESDAY

## PROGRAM – TUESDAY 25 JUNE 2019

16.30-17.00 Coffee Break & Exhibits **MODULE 8 & 7 1ST FLOOR**

17.00-18.00 **Presidential Lecture: Peripheral Neuropathy – 6th Edition** AUDITORIUM  
**Steven Scherer, MD, PhD, The University of Pennsylvania, Philadelphia, PA, USA**  
**Chair: Pieter Van Doorn, MD, PhD, President- Elect, Erasmus MC University Medical Centre, Rotterdam, Netherlands**



Steven S. Scherer is the Ruth Wagner Van Meter and J. Ray Van Meter Professor of Neurology at the University of Pennsylvania. He received his B.S., M.D., and Ph.D. from the University of Michigan, and did an internship, neurology residency, and a fellowship at the University of Pennsylvania, before joining the faculty in 1991. For 40 years, Dr. Scherer has sought to understand the causes and treatments of peripheral neuropathies, with a focus on inherited neuropathies in his clinical work and laboratory investigations. He has served on NIH Study Sections, on the medical board of the CMT Association, and is the current president of the Peripheral Nerve Society. Dr. Scherer was the first recipient of the Alan J. Gebhart – Peripheral Nerve Society Prize for Excellence in Peripheral Nerve Research.

18.00-18.30 **Awards & Prizes** AUDITORIUM

18.30-23.00 **Closing Reception** (Villa Lo Zerbino)



Before you depart, please join us for a spectacular PNS Closing Reception at the beautiful Villa Lo Zerbino. Buses depart from the main entrance of the convention centre and will arrive at the villa every 30 minutes. This event should not be missed!

Address: Passo dello Zerbino, 1, 16122 Genova GE, Italy

TUESDAY



Exhibitors

## GRIFOLS

### Grifols

TABLE #1

[www.grifols.com](http://www.grifols.com)  
[corporatecomms@grifols.com](mailto:corporatecomms@grifols.com)

For more than 75 years, Grifols has worked to improve the health and well being of people around the world. We are a global healthcare company that produces essential plasma-derived medicines for patients and provides hospitals and healthcare professionals with the tools, information and services they need to deliver expert medical care.



CHALLENGE ACCEPTED

### Alnylam Pharmaceuticals, Inc.

TABLE #2

[www.alnylam.com](http://www.alnylam.com)  
[info@alnylam.com](mailto:info@alnylam.com)

Alnylam is leading the translation of RNA interference into a new class of innovative medicines with the potential to transform the lives of people with rare genetic, cardio-metabolic, hepatic infectious, and CNS/ocular diseases. ONPATTRO® (patisiran) is Alnylam's first U.S. FDA-approved RNAi therapeutic, with five investigational medicines in late-stage development. [www.alnylam.com](http://www.alnylam.com).

## CSL Behring

Biotherapies for Life™

### CSL Behring

TABLE #3

[www.cslbehring.com](http://www.cslbehring.com)

CSL Behring is a global biotherapeutics leader driven by our promise to save lives. We meet patients' needs using the latest technologies to develop and deliver innovative biotherapies that are used to treat serious and rare conditions. Our Ig products treat debilitating diseases that range from immunologic, neurologic and vascular.



### Akcea: hATTR Amyloidosis with Polyneuropathy

TABLE #4

[www.akceatx.com](http://www.akceatx.com)  
[info@akceatx.com](mailto:info@akceatx.com)

Akcea Therapeutics, Inc., an affiliate of Ionis Pharmaceuticals, Inc. is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea is advancing a mature pipeline of six novel drugs. Additional information about Akcea is available at [www.akceatx.com](http://www.akceatx.com).



## Pfizer Inc.

### TABLE #5

[www.pfizer.com](http://www.pfizer.com)  
[info@pfizer.com](mailto:info@pfizer.com)

Pfizer Inc.: Working together for a healthier world® At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products.



## LFB BIOMEDICAMENTS

### TABLE #7

[www.groupe-lfb.com](http://www.groupe-lfb.com)  
[infomed@lfb.fr](mailto:infomed@lfb.fr)

Since it was formed in 1994, the LFB has been developing, manufacturing and marketing therapeutic proteins, for diseases that are always serious and often rare. The LFB is now one of the leading European players in biopharmaceutical, its products are aimed at health professionals, mainly hospital-based, these are plasma derived medicinal products in three major fields of therapy: immunology, haemostasis and intensive care.



## KEDRION BIOPHARMA

### TABLE #6

[www.kedrion.com](http://www.kedrion.com)  
[www.kedrion.com/kedrion-world](http://www.kedrion.com/kedrion-world)

KEDRION is an international company that collects and fractionates blood plasma to produce and distribute plasma-derived therapeutic products for use in treating serious diseases, disorders and conditions such as hemophilia and immune system deficiencies. A primarily family-owned business, it places a high value not only on the welfare of those who benefit from its products but also on the people and communities where it operates.



## PHARNEXT

### TABLE #8

[www.pharnext.com](http://www.pharnext.com)  
[contact@pharnext.com](mailto:contact@pharnext.com)

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases. Pharnext has two lead products in clinical development. PXT3003 completed an international Phase 3 trial with positive topline results in CMT1A and benefits from the orphan drug status. PXT864 generated encouraging Phase 2 results in Alzheimer's disease.

## EXHIBITOR DIRECTORY



### Takeda Pharmaceutical Company Limited

**TABLE #9**

[www.takeda.com](http://www.takeda.com)  
[www.takeda.com](http://www.takeda.com)

Takeda is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to bringing Better Health and a Brighter Future to patients by translating science into highly-innovative medicines. Takeda focuses its R&D efforts on four therapeutic areas: Oncology, Gastroenterology, Neuroscience and Rare Diseases and targeted R&D-investments in Plasma-Derived-Therapies and Vaccines.



### Takeda – Medical Affairs

**TABLE #12**

[www.takeda.com](http://www.takeda.com)  
[www.takeda.com](http://www.takeda.com)

Takeda is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to bringing Better Health and a Brighter Future to patients by translating science into highly-innovative medicines. Takeda focuses its R&D efforts on four therapeutic areas: Oncology, Gastroenterology, Neuroscience and Rare Diseases and targeted R&D-investments in Plasma-derived Therapies and Vaccines.



### Akcea: Global Medical Affairs

**TABLE #10**

[www.akceatx.com](http://www.akceatx.com)  
[info@akceatx.com](mailto:info@akceatx.com)

Akcea Therapeutics, Inc., an affiliate of Ionis Pharmaceuticals, Inc. is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea is advancing a mature pipeline of six novel drugs. Additional information about Akcea is available at [www.akceatx.com](http://www.akceatx.com).

## octapharma®

### Octapharma AG

TABLE #13

[www.octapharma.ch](http://www.octapharma.ch)  
[samira.hermann@octapharma.com](mailto:samira.hermann@octapharma.com)

About Octapharma: Octapharma is a global healthcare company developing and producing medicines based on human proteins from human plasma and human cell lines for three therapeutic areas:

- Haematology
- Immunotherapy
- Critical Care

Our products are available in 115 countries and reach hundreds of thousands of patients every year.

## disarm THERAPEUTICS

### Disarm

TABLE #14

[www.disarmtx.com/](http://www.disarmtx.com/)  
[info@disarmtx.com](mailto:info@disarmtx.com)

Disarm Therapeutics is creating breakthrough disease-modifying therapeutics that prevent axonal degeneration (AxD) for patients with neurological diseases. Our goal is to stop axon loss by inhibiting SARM1, the central driver of AxD in diseases of the central and peripheral nervous systems, including Multiple Sclerosis, ALS and peripheral neuropathies.

## Alnylam® PHARMACEUTICALS

CHALLENGE ACCEPTED

### Alnyam Pharmaceuticals Medical Information

TABLE #15

[www.alnylam.com](http://www.alnylam.com)  
[medinfo@alnylam.com](mailto:medinfo@alnylam.com)

Alnylam is leading the translation of RNA interference into a new class of innovative medicines with the potential to transform the lives of people with rare genetic, cardio-metabolic, hepatic infectious, and CNS/ocular diseases. ONPATTRO® (patisiran) is Alnylam's first U.S. FDA-approved RNAi therapeutic, with five investigational medicines in late-stage development. [www.alnylam.com](http://www.alnylam.com).

## TERUMOBCT

Unlocking the Potential of Blood

### Terumo BCT

TABLE #16

[www.terumobct.com](http://www.terumobct.com)  
[www.terumobct.com/contact-us](http://www.terumobct.com/contact-us)

Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We are committed to advancing apheresis through innovation in plasmapheresis using centrifugal technology. Our collaboration with customers helps enable the best possible procedures today, increase the number of clinicians who adopt these procedures, identify new uses for apheresis and develop next-generation procedures — all for the patients we ultimately serve.

# EXHIBITOR DIRECTORY



**UCB**

**TABLE #17**

[www.ucb.com](http://www.ucb.com)  
[contactucb@ucb.com](mailto:contactucb@ucb.com)

UCB is a global biopharmaceutical company with a focus on neurology and immunology. Our medicines treat thousands of people around the world. We engage with patients, families and healthcare professionals to address unmet needs. We are around 7500 people in all four corners of the globe, inspired by patients and driven by science.



**GBS|CIDP Foundation International**

**TABLE #18**

[www.gbs-cidp.org](http://www.gbs-cidp.org)  
[info@gbs-cidp.org](mailto:info@gbs-cidp.org)

The GBS|CIDP Foundation International is a global nonprofit organization supporting individuals and their families affected by Guillain-Barre Syndrome, chronic demyelinating polyneuropathy and related conditions through a commitment to provide support and education, fund research and to advocate for the patient voice.



**European Cancer  
Patient Coalition**

**European Cancer Patient Coalition**

**TABLE #19**

[www.ecpc.org](http://www.ecpc.org)  
[info@ecpc.org](mailto:info@ecpc.org)

European Cancer Patient Coalition (ECPC) is the largest European cancer patients' umbrella organisation. Established in 2003 with over 450 members, the ECPC is the voice of the European cancer community, uniquely representing the interests of all cancer patient groups, from the most common to the rarest forms of cancer.



**Hereditary Neuropathy Foundation**

**TABLE #20**

[www.hnf-cure.org](http://www.hnf-cure.org)

HNF, a CMT advocacy and research organization leads efforts in the development of breakthrough treatments with its many stakeholder partnerships. With a commitment to patient-focused research, HNF has been instrumental in hosting the groundbreaking FDA EI-PFDD meeting in 2018 and in the development of the first potential drug for CMT1A.



## ACMT-Rete per la Malattia di Charcot-Marie-Tooth

**TABLE #21**

[www.acmt-rete.it](http://www.acmt-rete.it)  
[acmt-rete@acmt-rete.it](mailto:acmt-rete@acmt-rete.it)

ACMT-Rete is an Italian not-for-profit organization with the following aims:

- Promoting information projects in order to increase awareness on the treatment of CMT disease and similar syndromes;
- Encouraging the creation of centers for diagnosis and treatment working in strict collaboration;
- Developing a relationship network across people suffering from Charcot-Marie-Tooth's disease.



## Amyloidosis Research Consortium

**TABLE #22**

[www.arci.org](http://www.arci.org)  
[arci@arci.org](mailto:arci@arci.org)

The Amyloidosis Research Consortium (ARC) is a global organization founded in 2015 dedicated to changing the way research is being done and focusing on what will have the greatest impact on improving the lives of patients. We forge collaborations across industry, academia and regulatory to improve and accelerate research.



## CMT Research Foundation

**TABLE #23**

[cmtrf.org](http://cmtrf.org)  
[info@cmtrf.org](mailto:info@cmtrf.org)

Focused on development of treatments and cures for CMT, The CMT Research Foundation funds the most promising, high-impact translational research by developing strategic alliances with academic and industry stakeholders. On a rolling basis, The CMT Research Foundation accepts innovative proposals which solve missing links and advances current research.



## European CMT Federation

**TABLE #24**

[ecmtf.org](http://ecmtf.org)  
[info@ecmtf.org](mailto:info@ecmtf.org)

The Federation of European Associations of patients suffering from de Charcot-Marie-tooth disease

## EXHIBITOR DIRECTORY



### Charcot-Marie-Tooth Association

#### TABLE #25

cmtausa.org  
info@cmtausa.org

The Charcot-Marie-Tooth Association is dedicated to finding treatments and a cure for CMT, and to improving the quality of life for everyone living with the disease. The CMTA's Strategy to Accelerate Research (STAR) brings top researchers together with pharmaceutical and biotechnology partners to accelerate scientific breakthroughs. STAR has one goal: to accelerate the development of treatments for CMT. Both research and treatment happen at the two dozen CMTA-supported Centers of Excellence at top-flight medical institutions nationwide. The CMTA also offers a number of community programs, including more than 70 local branches nationwide; Camp Footprint, the nation's only camp exclusively for kids with CMT; and Patient/Family conferences, where patients can meet the researchers and clinicians working on their behalf. To learn more about the CMTA, please visit [www.cmtausa.org](http://www.cmtausa.org).



### CMT United Kingdom

#### TABLE #26

cmt.org.uk  
cmt.office@cmt.org.uk

CMT United Kingdom is the charity working to support those who are affected by Charcot-Marie-Tooth Disease. We offer support to people who have Charcot-Marie-Tooth Disease and their careers, initiate, co-ordinate, promote and support research into CMT and its effects. Stimulate medical education on the subject of CMT. Spread awareness of the condition to health professionals and the general public.



### EPODIN / European Patient Organisation for Dysimmune and Inflammatory Neuropathies

#### TABLE #27

[www.epodin.org](http://www.epodin.org)  
contact@epodin.org

EPODIN is an International Non-Governmental Organization, composed of European patients' organizations involved in the field of rare immune-mediated peripheral-neuropathies (IMN). The contribution of EPODIN aims, by mobilizing and strengthening the patient's voice, to give each European citizen living with IMN the opportunity to reduce the burden and inequalities due to the disease.



**ENMC**  
**(European Neuromuscular Centre)**

**TABLE #28**

[www.enmc.org](http://www.enmc.org)  
[enmc@enmc.org](mailto:enmc@enmc.org)

The European Neuromuscular Centre (ENMC) was founded 25 years ago, with the aim to bring leading researchers, clinicians and patient representatives from all over the world together via the well-known platform of ENMC workshops. Topics of the workshops vary from diagnostic tools, outcome measures, clinical trial readiness, to care management for all neuromuscular conditions.



*the* FOUNDATION for  
 PERIPHERAL NEUROPATHY®

**The Foundation for  
 Peripheral Neuropathy**

**TABLE #29**

[www.foundationforpn.org](http://www.foundationforpn.org)  
[info@tffpn.org](mailto:info@tffpn.org)

The Foundation for Peripheral Neuropathy is a public, 501c(3) charity organization committed to fostering collaboration among today's most gifted and dedicated neuroscientists and physicians. The Foundation's mission is to dramatically improve the lives of people living with peripheral neuropathy by advancing research, raising awareness, accelerating a cure, funding efforts of leading scientists and serving as the premier resource of information for patients, their families and healthcare providers.



**Canè S.p.A. Medical Technology**

**TABLE #30**

[www.canespa.it](http://www.canespa.it)  
[mailbox@canespa.it](mailto:mailbox@canespa.it)

Canè S.p.A. Medical Technology : 40 years of Italian excellence and experience

Starting from the first syringe drivers, Canè's products have evolved into Crono® line of miniature pumps. Visit our website: [canespa.it](http://canespa.it).



# Takeda: a trusted partner in peripheral neuropathies

Takeda has a robust foundation of experience in treating rare immunological conditions. Built on this experience, the future of Takeda is dedicated to treatments for patients with selective peripheral neuropathies.

**Join our PNS symposium  
at 7:15 – 8:15 am on  
Monday 24 June in the  
Main Auditorium**



## Thirty years of MMN diagnosis and treatment: What does the future hold for our patients?

**With presentations from:**

**Prof. Leonard H. van den Berg**

University Medical Center Utrecht  
The Netherlands

**Prof. Eduardo Nobile-Orazio**

University of Milan  
Italy

**Prof. Michel Magistris**

Geneva University Hospitals  
Switzerland



## **ATTR POLYNEUROPATHY:** **The source, the impact, the management**

Sunday 23<sup>rd</sup> June 2019

07:15–08:15 AM

Levante/Ponente

Centro Congressi, Porto Antico di Genova, Genoa, Italy

Co-Chair: Dr Teresa Coelho (Portugal)

Co-Chair: Dr Laura Obici (Italy)

### **Agenda**

<b>Time</b>	<b>Session</b>	<b>Faculty</b>
07:15–07:20	<b>Welcome and introduction</b>	Dr Teresa Coelho (Portugal) and Dr Laura Obici (Italy)
07:20–07:35	<b>TTR in health and disease: What role does it really play?</b>	Dr Laura Obici (Italy)
07:35–07:50	<b>Clinical follow up of asymptomatic gene carriers: Achieving the earliest diagnosis and therapeutic intervention</b>	Dr Isabel Conceição (Portugal)
07:50–08:05	<b>The patient journey: Improving care through a personalised approach to follow up</b>	Dr Teresa Coelho (Portugal)
08:05–08:10	<b>Audience Q&amp;A</b>	All faculty Moderated by Co-Chairs
08:10–08:15	<b>Summary and close</b>	Dr Teresa Coelho (Portugal) and Dr Laura Obici (Italy)



# Pushing the boundaries of science

Everything we do starts with one simple question:

**“ how will this make a difference to the lives of patients? ”**

UCB has a passionate, long-term commitment to help patients and families living with severe diseases lead normal, everyday lives.

Our ambition is to offer them innovative new medicines and ground-breaking solutions in two main therapeutic areas: neurology and immunology. We foster cutting-edge scientific research that is guided by patients' needs.

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congress

Oslo  
2019

5<sup>th</sup> Congress of the  
European Academy  
of Neurology

June 29 – July 2

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sees  
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[www.ean.org/oslo2019](http://www.ean.org/oslo2019)  
#ean2019

ean  
congress

Paris  
2020

6<sup>th</sup> Congress of the  
European Academy  
of Neurology

May 23 – 26

A  
bientôt  
à Paris

Abstract submission  
deadline  
08 January 2020

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#ean2020

# A visionary concept in drug development



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## PLEOTHERAPY™

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based on big-data genomics and artificial intelligence



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2019 PNS Annual Meeting

# A '3D look' at hATTR Amyloidosis: Diagnosis, Disease progression and Defining real-world treatment outcomes

Monday, June 24, 2019

19:00–20:00 | Grecale Room

Centro Congressi – Porto Antico di Genova  
Genoa, Italy

## CHAIR



**Teresa Coelho, MD**

Santo António Hospital, Porto, Portugal

## SPEAKERS



**Christopher J. Klein, MD**

Mayo Clinic, Rochester, Minnesota, USA



**Laura Obici, MD**

University of Pavia, Pavia, Italy

## OVERVIEW

Join Professor Teresa Coelho and our expert faculty panel for this interactive debate-style symposium which will seek to answer key clinical questions around hATTR amyloidosis and the critical role peripheral nerve specialists play in the management of these patients:

- How do we rapidly diagnose the disease and evaluate polyneuropathy impairment?
- What role does genetic testing have in screening patients in high-risk populations?
- How do we define disease progression?
- Can we predict treatment failure?
- How do we assess disease progression in the real world?

ONPATTRO<sup>®</sup>▼ (patisiran) is indicated for the treatment of hATTR amyloidosis in adult patients with Stage 1 or Stage 2 polyneuropathy<sup>1</sup>



onpattro<sup>®</sup>▼  
2 mg/mL concentrate for solution  
for infusion patisiran

- Through a natural process called RNAi, patisiran causes the catalytic degradation of TTR mRNA in the liver, resulting in a reduction of serum TTR protein<sup>1</sup>
- 56.1% of ONPATTRO<sup>®</sup>-treated patients *versus* 3.9% of placebo-treated patients experienced improvements in mNIS+7<sup>a</sup> ( $p < 0.001$ )<sup>1 b</sup>
- At 18 months, a benefit with ONPATTRO<sup>®</sup> to placebo was observed across all domains of Norfolk QoL-DN<sup>1</sup>

## To learn more please visit the Alnylam booth

Please request the full Summary of Product Characteristics at the Alnylam booth for further details  
ONPATTRO<sup>®</sup> is currently not commercially available in Italy, pending pricing and reimbursement approvals

<sup>a</sup>Composite measure of motor, sensory, and autonomic polyneuropathy

<sup>b</sup>In a threshold analysis of mNIS+7 (change from baseline of <0 points)

ATTR, TTR amyloidosis; hATTR, hereditary ATTR (hATTR) amyloidosis; mNIS+7, modified NIS plus 7; mRNA, messenger RNA;

NIS, neuropathy impairment score; QoL-DN, Quality of Life-Diabetic Neuropathy; RNA, ribonucleic acid; RNAi, RNA interference; TTR, transthyretin

Developed and produced by Alnylam Pharmaceuticals

### Reference:

1. Alnylam Pharmaceuticals. ONPATTRO<sup>®</sup>. EU Summary of Product Characteristics, 2018



Alnylam Netherlands B.V.  
Strawinskylaan 3051  
1077ZX Amsterdam  
Netherlands



This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system, by contacting the Rete Nazionale di Farmacovigilanza dell' Agenzia Italiana del Farmaco ([www.agenziafarmaco.gov.it/content/come-segnalare-una-sospetta-reazione-avversa](http://www.agenziafarmaco.gov.it/content/come-segnalare-una-sospetta-reazione-avversa))

ONPATTRO<sup>®</sup> is a trademark of Alnylam Pharmaceuticals, Inc.

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For chronic inflammatory demyelinating polyneuropathy (CIDP)

# FOLLOW THE PATH

The next step in CIDP treatment

Visit CSL Behring at table #3

## “Follow the PATH” to the CSL Behring table to learn about:

- PATH, the largest CIDP clinical trial
- The first and only subcutaneous Ig (SCIG) for maintenance treatment in CIDP
- CSL Behring and the 100+ years of experience developing and delivering plasma-derived products

### Attend our Symposium:

**Time:** Sunday, 23 June 2019 at 19:00

**Location:** Maestrone Auditorium

**Speakers:** David Cornblath, MD (Chair)  
Jeffrey Allen, MD  
Pieter van Doorn, MD, PhD  
Peter Van den Bergh, MD, PhD

**Theme:** Navigating diagnosis and treatment challenges in CIDP

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[www.CSLBehring.com](http://www.CSLBehring.com) ITA-HIZ-0004  
Submitted to AIFA on 09/05/2019

CIDP indication currently not reimbursed in Italy for Hizentra.  
Please see Italian SPC available at table.

A middle-aged couple is shown in a close embrace, smiling warmly. The man, on the left, has grey hair and a beard, wearing an orange short-sleeved button-down shirt. The woman, on the right, has blonde hair and is wearing a grey long-sleeved top. They are standing outdoors with a bright, slightly hazy sky in the background. A large dark blue square is positioned in the top left corner of the page.

# CARING FOR PEOPLE

For more than 75 years, Grifols has worked to improve the health and well-being of people around the world. We are committed to producing essential plasma-derived medicines for patients and to providing hospitals, pharmacies, and healthcare professionals with the tools, information, and services they need to deliver expert medical care.

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pioneering spirit

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[www.grifols.com](http://www.grifols.com)

**25<sup>th</sup> JUNE**  
**7:15 am**

# EVIDENCE-BASED MEDICINE AND CURRENT ADVANCES IN MULTIFOCAL MOTOR NEUROPATHY

## **CO-CHAIRS:**

Dr Jean-Marc Léger, Pr Hans Peter Hartung

Epidemiology, pathophysiology and new perspectives in Multifocal Motor Neuropathy: an update

**Pr Hans Peter Hartung - Germany (15')**

Multifocal Motor Neuropathy: diagnosis and treatment monitoring

**Pr Yusuf Rajabally - UK (15')**

A randomized comparative trial with 2 IVIGs in Multifocal Motor Neuropathy: the LIME study results

**Dr Jean-Marc Léger - France (15')**

5' wrap up

## Immunomodulation therapy in CIDP<sup>1</sup> & GBS<sup>2</sup>

Intravenous  
human normal immunoglobulin (IVIG)

**octagam®5%**<sup>1,2</sup>

50 mg/mL solution for infusion

**octagam®10%**<sup>1,2</sup>

100 mg/mL solution for infusion

**panzyga®**▼<sup>2</sup>

100 mg/mL solution for infusion

▼ This medicinal product is subject to additional monitoring.

The Summary of Product Characteristics of octagam®5%, octagam®10% and panzyga® are available on request at the booth. The products are licensed in Europe for immunomodulation in adults, and children and adolescents (0-18 years) in GBS (indicated as "2"), whereas octagam®5% and octagam®10% are also licensed for CIDP (indicated as "1"). In Italy, octagam®10% is licensed as Gamten® and panzyga® as Globiga®.

Abbreviations: CIDP, chronic inflammatory demyelinating polyneuropathy; GBS, Guillain-Barré syndrome.

## EXPLORE GENOA

### GENOA MAP

Stop by the Convention Visitors Bureau desk in the Ground Floor Lobby to pick up a map of the local area including locations for conference events and metro stations.

#### Desk Hours:

Saturday, 22 June	9.00 – 18.00
Sunday, 23 June	9.00 – 18.00
Monday, 24 June	9.00 – 18.00
Tuesday, 25 June	9.00 – 18.00

### ONLINE

To discover more about Genoa, local attractions and events happening during your stay, go to [www.visitgenoa.it/en](http://www.visitgenoa.it/en).

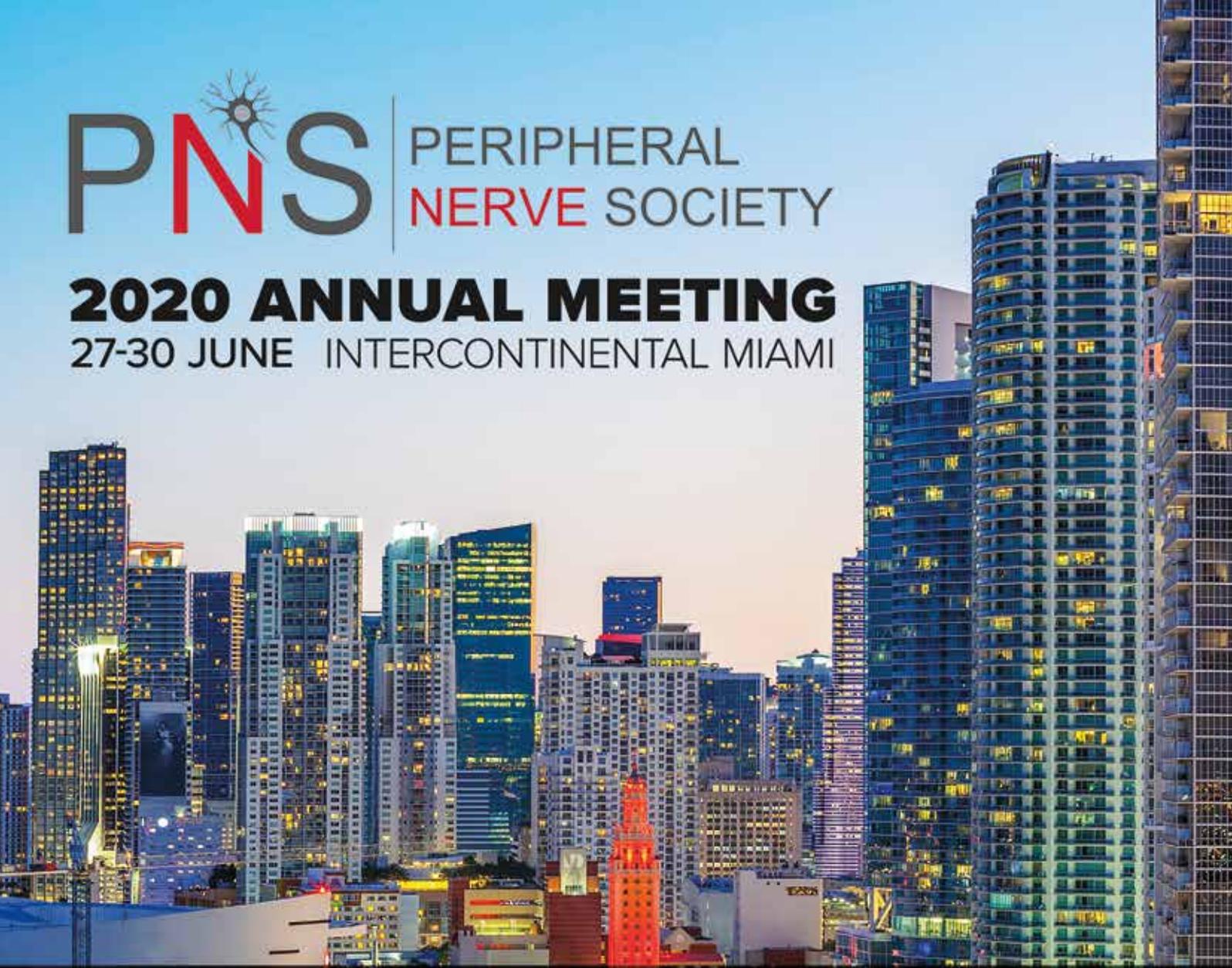






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**2020 ANNUAL MEETING**  
27-30 JUNE INTERCONTINENTAL MIAMI



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