

P R O G R A M



PNS ANNUAL MEETING

2018

BALTIMORE

M A R Y L A N D

21-25 JULY

Renaissance Baltimore Harborplace Hotel



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www.pnsociety.com



Program

2018
PNS Annual Meeting

21-25 July
Baltimore, MD, USA

WELCOME



On Behalf of the Peripheral Nerve Society, we are delighted to welcome you to the 2018 Annual Meeting at the Renaissance Baltimore Harborplace Hotel in Baltimore, Maryland, USA.

The PNS Annual Meeting continues to be the premier meeting for cutting-edge innovation and advances in peripheral neuropathy. The 2018 meeting will provide the usual mixture of excellent plenary lectures, oral platforms, oral posters and poster sessions. This year we have added parallel sessions on Sunday and Monday afternoon organized by special interest groups Inflammatory Neuropathy Consortium (INC), Charcot-Marie-Tooth and Related Neuropathies Consortium (CMTR) and the Diabetic Neuropathy Consortium. There will also be dedicated clinical trials sessions. We look forward to you being part of it.

Please join us for the Opening Ceremony on Saturday evening at the Maryland Science Center. The reception will feature complimentary drinks and hors d'oeuvres.

Coffee breaks and lunch will be provided daily for delegates. Please see the program for additional details. Complimentary internet will also be provided. Instructions for access can be found on page 10 of the program.

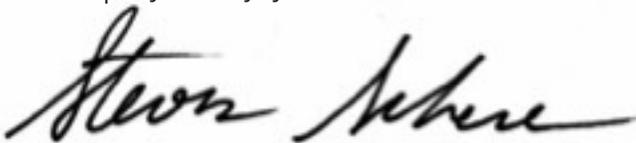
Be sure to attend the PNS Annual General Meeting on Monday at 12:30 in Maryland Ballroom ABCD. We will be reviewing Society business, and your input is needed. All are encouraged to attend.

Sunday night, all are invited to join, as we honor Junior and new Members of the Society with a reception at the hotel. Monday evening, the PNS will have another all member reception, to maximize your networking time with your colleagues.

Before you depart, please join us for a unique and fun PNS Closing Reception at Camden Yards, where we will watch an Orioles vs Red Sox Baseball Game. Transportation will be provided to and from the hotel.

I want to personally thank the Scientific Program Committee, PNS Board, Meeting Faculty, and everyone else who has generously donated their time and efforts to put together this outstanding meeting.

I hope you enjoy the 2018 PNS Annual Meeting and your stay in Baltimore.



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

OFFICERS

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Why Join: We are an international organization of physicians and scientists working together to develop and provide the best treatments for people who have peripheral nerve diseases. This goal is realized by cooperation- supporting research, training scientists, physicians and other healthcare professionals, setting standards of care, creating new treatments, and facilitating clinical trials.

Networking. Becoming a member of the PNS means collaborating with prominent global scientists, physicians and other healthcare professionals, setting standards of care, creating new treatments and facilitating clinical trials.

Education. PNS provides members with direct access to the JPNS, discounted Annual Meeting registration, guidelines, the latest updates on what's happening in the field, and other resources to aid in the education of members.

What to expect from your new membership?

- Access to Member Directory
- Journal of the Peripheral Nervous System (JPNS)
- Voting Rights
- Patient Information
- Career Planning
- Volunteer Opportunities
- Discounted Annual Meeting Rates
- PNS Members may join SIG groups upon request.

Visit www.pnsociety.com to Join Today!

The Peripheral Nerve Society 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 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

info@PNSociety.com | www.pnsociety.com | +1-952-545-6284

PNS – EXECUTIVE OFFICE

Janel Fick, Executive Director
Allison Kindseth, Associate Executive Director
Tanya Baker, Meetings & Engagement Manager

PAST MEETINGS OF THE PNS

- 2017 Sitges-Barcelona, Spain
- 2015 Quebec City, Canada
- 2013 Saint-Malo, France
- 2011 Potomac, Maryland
- 2009 Würzburg, Germany
- 2007 Snowbird, Utah
- 2005 Tuscany, Italy
- 2003 Banff, Canada
- 2001 Tyrol, Austria
- 1999 La Jolla, California
- 1997 Cambridge, England
- 1995 Antalya, Turkey
- 1994 Saint Paul, Minnesota

PAST MEETINGS OF THE PNSG

- 1993 Boppard, Germany
- 1991 Arden House, New York
- 1989 Padua, Italy
- 1987 Lake Couchichinh, Ontario, Canada
- 1985 Mürren, Switzerland
- 1983 Fontevraud, France
- 1981 Shakertown, Kentucky
- 1979 Wye College, Kent, England
- 1977 Airlie House, Virginia
- 1975 Rochester, Minnesota
- 1974 Carville, Louisiana

PAST MEETINGS OF THE PNA AND PNA

- 1992 Rapallo, Italy
- 1990 Oxford, England
- 1989 Maui, Hawaii
- 1988 Halifax, Nova Scotia, Canada
- 1986 Hilton Head Island, South Carolina
- 1985 Keystone, Colorado
- 1984 Keystone, Colorado

PROGRAM AT A GLANCE

SATURDAY 21 JULY 2018

- 8.00-12.00 Individual Meeting: Inherited Neuropathy Consortium ([HOMELAND](#))
- 9.00-18.00 Education Course ([MARYLAND BALLROOM ABCD](#))
- 18.00-21.00 Welcome Reception ([MARYLAND SCIENCE CENTER INNER HARBOR](#))

SUNDAY 22 JULY 2018

- 7.30-8.30 Light Breakfast & Poster Viewing ([BALTIMORE BALLROOM A&B](#))
- 8.30-9.00 Plenary Session: Richard Bunge Lecture ([MARYLAND BALLROOM A-D](#))
- 9.00-10.30 Platform Session I ([MARYLAND BALLROOM A-D](#))
- 10.30-11.00 Coffee Break ([MARYLAND FOYER](#))
- 11.00-11.30 Plenary Session: Peter J Dyck Lecture ([MARYLAND BALLROOM A-D](#))
- 11.30-12.30 Oral Poster Session I ([MARYLAND BALLROOM A-D](#))
- 12.30-13.00 Lunch & Poster Viewing ([BALTIMORE BALLROOM A&B](#))
- 12.30-13.00 Individual Meeting: IMAGiNe Study ([MARYLAND BALLROOM EF](#))
- 13.00-14.00 Poster Session I ([MARYLAND BALLROOM A-D](#))
- 14.00-18.00 Special Interest Group (SIG) Parallel Sessions
- INFLAMMATORY NEUROPATHY CONSORTIUM (INC) ([MARYLAND BALLROOM A-D](#))
 - CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM ([MARYLAND BALLROOM E&F](#))
 - DIABETES/PAIN CONSORTIUM ([WATERTABLE BALLROOM](#))
- 16.00-16.30 Coffee Break ([MARYLAND FOYER](#))
- 18.00-19.00 Sponsored Symposia
- AKCEA Symposium ([MARYLAND BALLROOM E&F](#))
 - CSL Behring Symposium ([MARYLAND BALLROOM A-D](#))
- 19.00-20.00 Junior Reception ([MARYLAND FOYER](#))

MONDAY 23 JULY 2018

- 7.30-8.30 Light Breakfast & Poster Viewing ([BALTIMORE BALLROOM A&B](#))
- 8.30-9.00 Plenary Session: Arthur K Asbury Lecture ([MARYLAND BALLROOM A-D](#))
- 9.00-10.30 Platform Session II ([MARYLAND BALLROOM A-D](#))
- 10.30-11.00 Coffee Break ([MARYLAND FOYER](#))
- 11.00-11.30 Plenary Session: PK Thomas Lecture ([MARYLAND BALLROOM A-D](#))

- 11.30-12.30 Oral Poster Session II ([MARYLAND BALLROOM A-D](#))
- 12.30-13.00 PNS Annual General Meeting (all are welcome) ([MARYLAND BALLROOM A-D](#))
- 13.00-14.00 Lunch & Poster Session II ([BALTIMORE BALLROOM A&B](#))
- 14.00-18.00 Special Interest Group (SIG) Parallel Sessions
- INFLAMMATORY NEUROPATHY CONSORTIUM (INC) ([MARYLAND BALLROOM A-D](#))
 - CHARCOT-MARIE-TOOTH & RELATED NEUROPATHIES CONSORTIUM ([MARYLAND BALLROOM E](#))
 - DIABETES/PAIN CONSORTIUM ([WATERTABLE BALLROOM](#))
- 16.00-16.30 Coffee Break ([MARYLAND FOYER](#))
- 18.00-19.00 Sponsored Symposia
- Alnylam Symposium ([MARYLAND BALLROOM E&F](#))
 - Grifols Symposium ([MARYLAND BALLROOM A-D](#))
- 19.00-20.00 All Member Reception ([MARYLAND FOYER](#))

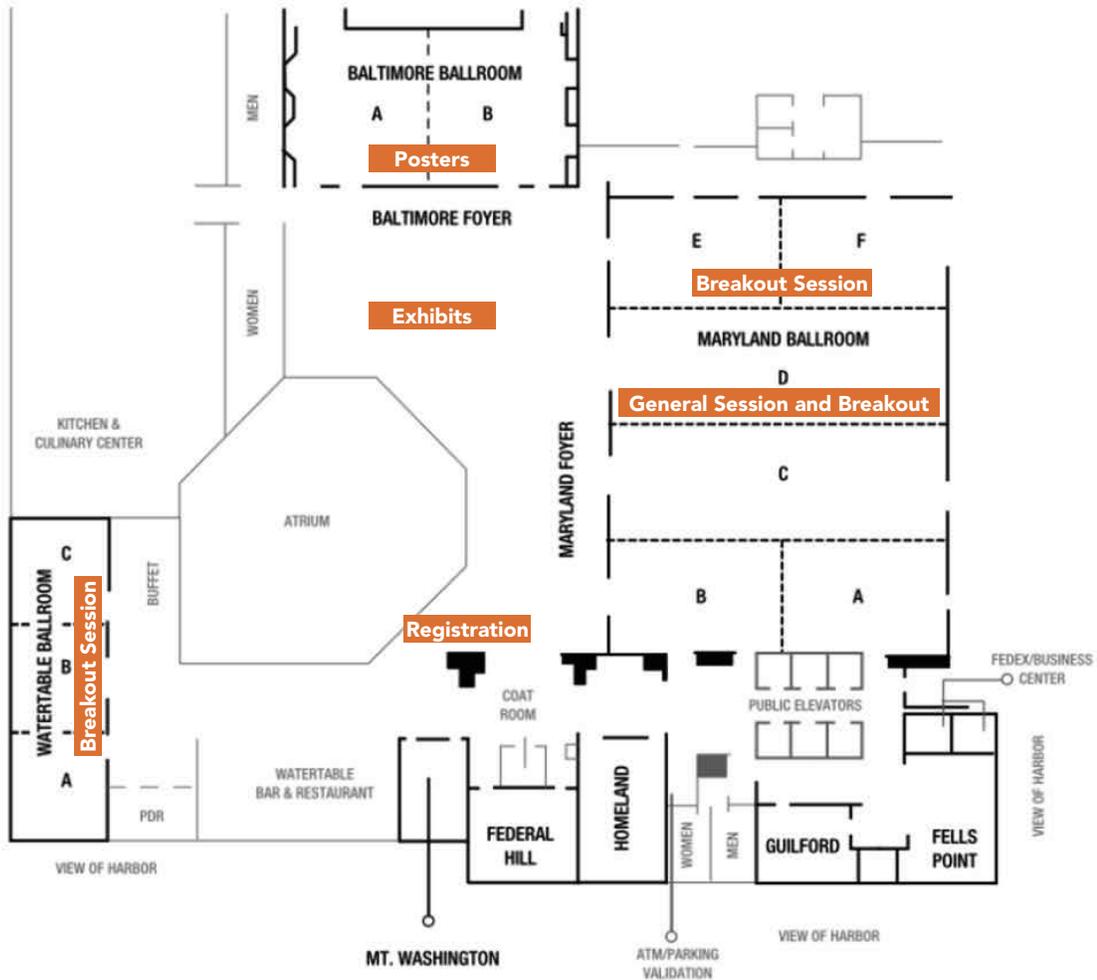
TUESDAY 24 JULY 2018

- 7.30-8.30 Light Breakfast & Poster Viewing ([BALTIMORE BALLROOM A&B](#))
- 8.30-9.00 Plenary Session: Jack Griffin Lecture ([MARYLAND BALLROOM A-D](#))
- 9.00-10.30 Platform Session III ([MARYLAND BALLROOM A-D](#))
- 10.30-11.00 Coffee Break ([MARYLAND FOYER](#))
- 11.00-11.30 Plenary Session: Pembroke Lecture
- Cell Biology ([MARYLAND BALLROOM A-D](#))
- 11.30-12.30 Oral Poster Session III ([MARYLAND BALLROOM A-D](#))
- 13.00-14.00 Lunch & Poster Session III ([BALTIMORE BALLROOM A&B](#))
- 14.00-16.00 Clinical Trials Updates and Late Breaking Abstract ([MARYLAND BALLROOM A-D](#))
- 16.00-16.30 Coffee Break ([MARYLAND FOYER](#))
- 16.30-17.30 Closing Keynote
- Arthur Burghes ([MARYLAND BALLROOM A-D](#))
- 17.30-18.00 Awards & Prizes ([MARYLAND BALLROOM A-D](#))
- 18.30 Closing Reception at Camden Yards Orioles vs. Red Sox Baseball Game
- Buses depart from main hotel entrance*

WEDNESDAY 25 JULY 2018

- 9.00-11.00 Special Interest Group (SIG): Toxic Neuropathy ([MARYLAND BALLROOM EF](#))

FIFTH FLOOR



SIXTH FLOOR



GENERAL INFORMATION

REGISTRATION / HELP DESK HOURS

Friday, 20 July	17.30 – 19.30
Saturday, 21 July	7.30 – 18.00
Sunday, 22 July	7.30 – 18.00
Monday, 23 July	7.30 – 18.00
Tuesday, 24 July	7.30 – 18.00

REGISTRATION FEES

Registration fees include:

- Access to all conference sessions
- Breakfast, coffee breaks, and lunch
- Welcome Reception at the Maryland Science Center
- Junior Reception (all are welcome)
- All Member Reception
- Closing Session at the Camden Yards Baseball Field

BADGE

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

CERTIFICATE OF ATTENDANCE

Your certificate may be downloaded from the PNS website after the meeting.

WIFI INSTRUCTIONS

Complimentary WIFI is available in the meeting space.

Network: PNS2018

Password: pns2018

MOBILE APP INSTRUCTIONS

(use this to participate with the audience response system)

Navigate the event like a pro with the PNS 2018 Annual Meeting mobile app, powered by Core-apps.

With the PNS 2018 Annual Meeting app, you can:

- Stay organized with up-to-the-minute speaker, exhibitor and event information
- Receive important real-time communications from PNS
- Build a personalized schedule and bookmark exhibitors
- Take notes and download event handouts and presentations
- Find attendees and connect with your colleagues through Friends
- Stay in-the-know and join in on social media with #PNS2018
- Share your event photos and experience with the Photo Gallery
- And much, much more!

DOWNLOADING THE APP IS EASY!

While on your smartphone, point your mobile browser to <http://app.core-apps.com/pnsm2018> to be directed to the proper download version for your device.

EXHIBITION

Please be sure to visit the exhibits located in the foyer at the breaks.

LIABILITY & INSURANCE

The organizers accept no responsibility for any injury or damage involving persons and property during the 2018 PNS Meeting.

POSTER SESSIONS

POSTER SESSION I: Sunday 22 July
Room: Baltimore Ballroom A&B
Mandatory Stand by Time: 13.00-14.00

POSTER SESSION II: Monday 23 July
Room: Baltimore Ballroom A&B
Mandatory Stand by Time: 13.00-14.00

POSTER SESSION III: Tuesday 24 July
Room: Baltimore Ballroom A&B
Mandatory Stand by Time: 13.00-14.00

ACKNOWLEDGMENTS

*We thank the generosity of our sponsors.
This program would not be possible without their support.*

YEAR ROUND PARTNERS



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Please reference page 37 for the exhibitor directory.

GENERAL SPONSORS



PATIENT ADVOCACY GROUPS (PAGS)







- 07.30-18.00** Registration / Help Desk Open (MARYLAND FOYER)
- 8.00-12.00** **Individual Meeting: Inherited Neuropathy Consortium** (HOMELAND)
Hosted by: **Michael Shy**
- 09.00-18.00** **Education Course** (MARYLAND BALLROOM ABCD)
Chairs: **Alex Rossor** and **Alessandra Bolino**
- 09.00-09.45** An Overview of Peripheral Nerve Anatomy and Pathology
Steven Scherer
- 09.45-10.30** Disease Mechanisms in the Inherited Neuropathies
Vincent Timmerman
- 10.30-11.00** Coffee Break (MARYLAND FOYER)
- Education Course Continued
Chairs: **Michelle Mauerman** and **Ruth Huizinga**
- 11.00-11.45** New Antibodies and Mechanisms in Immune Mediated Neuropathy
Claudia Sommer
- 11.45-12.30** How I Treat Paraproteinaemic Neuropathies
Michelle Mauermann
- 12.30-14.00** Lunch (MARYLAND FOYER)
- Education Course Continued
Chairs: **Josh Burns** and **Davide Pareyson**
- 14.00-14.40** Genetic Therapies in CMT and Related Disorders, How They Work and Clinical Applications
John Svaren
- 14.40-15.20** The Journey from Preclinical to Clinical Trial: Lessons Learnt
Davide Pareyson
- 15.20-16.00** What Makes a Good Outcome Measure and How to Design One
Josh Burns
- 16.00-16.30** Coffee Break (MARYLAND FOYER)

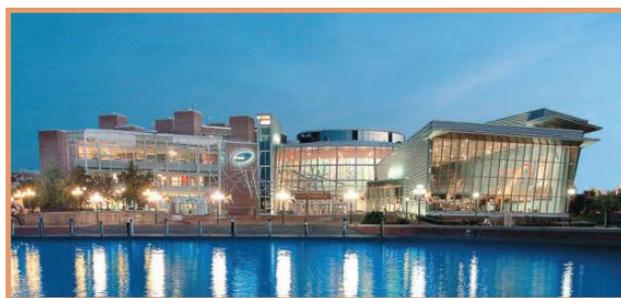
PROGRAM – SATURDAY 21 JULY 2018

Education Course Continued

Chairs: **Eduardo Nobile-Orazio** and **Pedro Tomaselli**

- 16.30-18.00** **Case Presentation Competition**
(Complete abstract details may be found in the abstract supplement)
- 16.30-16.45** Mononeuritis multiplex secondary to Lyme Neuroborreliosis
Chinar Osman, *Wessex Neurological Centre*
- 16.45-17.00** Early onset neuropathy with episodes of worsening and brown urine: Mitochondrial trifunctional protein (MTP) deficiency
Vanessa Marques, *School of Medicine of Ribeirão Preto - University of São Paulo*
- 17.00-17.15** Transthyretin Val107 mutation mimicking CIDP
Francisco de Assis Gondim, *Universidade Federal do Ceará*
- 17.15-17.30** Two cases of CIDP after Zika virus infection
Sonja Leonhard **Department of Neurology**, *Erasmus Medical Centre Rotterdam, Netherlands*
- 17.30-17.45** The intolerable endotracheal tube
Ludwig Gutmann, *University of Iowa*
- 17.45-18.00** PNS Masters Education Course - Milan, Italy
Giuseppe Lauria
- 18.30-21.00** **Welcome Reception** (MARYLAND SCIENCE CENTER INNER HARBOR)
The 2018 PNS Welcome Reception will kick off at the Maryland Science Center. Please join us in the inner harbor directly across from the hotel for amazing views, scientific activities, appetizers and a hosted bar. Directionals will be located outside of the hotel to help you find your way.

Maryland Science Center is located at 601 Light St, Baltimore, MD 21230





- 07.30-18.00** Registration / Help Desk Open (MARYLAND FOYER)
- 07.30-08.30** Light Breakfast & Poster Viewing (BALTIMORE BALLROOM AB)
- 08.30-09.00** **Plenary Session: Richard Bunge Lecture** (MARYLAND BALLROOM ABCD)
Introduction by **Ludo Van Den Bosch**



Autophagy Dynamics in Neuronal Homeostasis and Neurodegeneration

Erika Holzbaur, *Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA, USA*

- 09.00-10.30** **Platform Session I** (MARYLAND BALLROOM ABCD)
Chairs: **Haesun Kim** and **Mario Saporta**
(Complete abstract detail may be found in the abstract supplement.)
- 09.00-09.15** Deacetylation E3-Ubiquitin Protein Ligase by Sirtuin 1 Over Expression Reverses T2D Peripheral Neuropathy, **Krish Chandrasekaran**, *University of Maryland School of Medicine*
- 09.15-09.30** Activation of the ER Stress Transcriptions Factor XBP1 Modulates Disease Severity in MT1B Mice, **Maurizio D'Antonio**, *Division of Genetics and Cell Biology, San Raffaele Scientific Institute*
- 09.30-09.45** CRISPR/Cas9 mediated PMP22 downregulation improves the phenotype in a rodent model of CMT1A, **Ji-Su Lee**, *Department of Health Sciences and Technology, SAHST, Sungkyunkwan University, Korea*
- 09.45-10.00** Insights into Bortezomib-Induced Neurotoxicity Using Proteomics in Human Pluripotent Stem Cell-Derived Sensory Neurons, **Sybil Hrstka**, *Mayo Clinic*
- 10.00-10.15** Restoring Mitofusin Balance in the Nervous System to Prevent Axonal Degeneration from Mutant MFN2, **Yueqin Zhou**, *Center of Neural Science and Medicine, Cedars-Sinai Medical Center*
- 10.30-11.00** Coffee Break (MARYLAND FOYER)

PROGRAM – SUNDAY 22 JULY 2018

11.00-11.30 **Plenary Session: Peter J Dyck Lecture** (MARYLAND BALLROOM ABCD)
Introduction by **Ahmet Hoke**



Diabetes and Clinical Topics
Peter McNaughton, *Kings College London, London, United Kingdom*

11.30-12.30 **Oral Poster Session I** (MARYLAND BALLROOM ABCD)
Chairs: **Daniela Menichella** and **Maurizio D'Antonio**
(Complete abstract details may be found in the abstract supplement)

- 11.30-11.35** A kinase inhibitor improves neurofilament distribution in CMT2E human motor neuron axons, **Mario Saporta**, *University of Miami, Florida*
- 11.35-11.40** Impact of Patisiran on Overall Health Status in hATTR Amyloidosis: Results from the APOLLO Trial, **Senda Ajroud-Driss**, *Northwestern University Feinberg School of Medicine*
- 11.40-11.45** Mitochondria–Lysosome Contacts Regulate Mitochondrial Dynamics via GTP Hydrolysis of Rab7 Linked to Charcot-Marie-Tooth 2B, **Yvette Wong**, *Northwestern University Feinberg School of Medicine*
- 11.45-11.50** CMT2, CRISPR and Stem Cells: Disease Mechanism and Genome Surgery, **Bruce Conklin**, *Gladstone Institutes, UCSF, Innovative Genomics Institute*
- 11.50-11.55** Deletion of SARM1 has a Protective Effect for High-fat Diet-induced Peripheral Neuropathy and Glucose Intolerance, **Jun-Soon Kim**, *Johns Hopkins School of Medicine*
- 11.55-12.00** Specific Contribution of Distinct Dorsal Root Ganglion Neuron Subtypes To Painful Diabetic Neuropathy, **Nirupa Jayaraj**, *Northwestern University*
- 12.00-12.05** The Construct Validity and Diagnostic Capability of Patient and Clinician Reported Measures of CIPN, **Noah Kolb**, *University of Vermont*
- 12.05-12.10** Molecular signatures of microangiopathy in nerve pathology of diabetic neuropathy, **Sung-Tsang Hsieh**, *National Taiwan University Hospital*
- 12.10-12.15** AAV Targeting of Peripheral Nervous System Dysfunction in Giant Axonal Neuropathy, **Rachel Bailey**, *University of North Carolina at Chapel Hill*



- 12.15-12.20** New Role of Sirt2 in Alleviating Cisplatin Induced Peripheral Neuropathy Pain, **Manchao Zhang**, *Department of Radiation Oncology, The University of Arkansas for Medical Sciences*
- 12.30-13.00** Lunch & Poster Viewing (BALTIMORE BALLROOM AB)
- 12.30-13.30** **Individual Meeting: IMAGiNE Study** (MARYLAND BALLROOM EF)
Hosted by: **Ingemar Merkies**
- 13.00-14.00** **Poster Session I** (BALTIMORE BALLROOM AB)
All presenters are required to stand by posters from 13.00 - 14.00.
- 14.00-18.00** **Special Interest Group (SIG) Parallel Sessions**
- 14.00-18.00** ▶ **Inflammatory Neuropathy Consortium (INC)** (MARYLAND BALLROOM ABCD)
- 14.00-14.30** INC Business and Reviews of New and Ongoing Projects Welcome and Introduction:
Richard Lewis, *INC Chair*
- 14.00-14.10** Update on IGOS
Bart Jacobs
- 14.10-14.30** Vasculitic neuropathy - registry and RODS scale development
Robert Hadden and **Mike Collins**
- 14.30-15.00** Invited Talk: Experience with Diagnosis and Treatment of POEMS syndrome
Satoshi Kuwabara
Chair: **Chiara Briani**
- 15.00-16.00** **Oral Abstracts**
Chairs: **Carina Bunschoten** and **Janel Femi**
(Complete abstract details may be found in the abstract supplement)
- 15.00-15.10** Nerve Ultrasound for the Identification of Treatment-responsive Chronic Neuropathies without Nerve Conduction Abnormalities, **Stephan Goedee**, *Brain Center Rudolf Magnus, UMC Utrecht*
- 15.10-15.20** The safety and efficacy of thalidomide treatment prior to autologous stem-cell transplantation in POEMS syndrome, **Sonoko Misawa**, *Chiba University Graduate School of Medicine*
- 15.20-15.30** Intravenous immunoglobulin for mild Guillain-Barré syndrome: an international prospective observational study, **Joyce Roodbol**, *Erasmus MC*
- 15.30-15.40** Varied Phenotype and Treatment Responsiveness of Immune Checkpoint Inhibitor Associated Neuropathies: Report of 14 Patients, **Divyanshu Dubey**, *Brigham and Women's Hospital and Massachusetts General Hospital, and Mayo Clinic*

PROGRAM – SUNDAY 22 JULY 2018

SUNDAY

15.40-15.50 Innate Sensing of Viruses in the Guillain-Barré Syndrome, **Ruth Huizinga**, *Erasmus MC, University Medical Centre*

16.00-16.30 Coffee Break (*MARYLAND FOYER*)

16.30-17.00 Debates: The Next Best Clinical Trial in CIDP: B-Cell or T-Cell Immunomodulating Therapy?
Bernd Kieseier and **Mazen Dimachkie**
Introduction by **Luis Querol**

17.00-17.30 Mechanisms of IVIG and SCIG Effectiveness in Immune Neuropathies
Mel Berger
Chair: **Thomas Harbo**

17.30-18.00 **Oral Abstracts**
Chair: **Judith Spies**
(Complete abstract details may be found in the abstract supplement)

17.30-17.40 Comparison of Guillain-Barre Syndrome Between the Epidemic and Post-Epidemic Phases of Zika Virus in Colombia, **Laura Munoz**, *Johns Hopkins Hospital*

17.40-17.50 Virus discovery in chronic inflammatory demyelinating polyneuropathy, **Gwen van Lieferloo**, *Academic Medical Center, Department of Neurology*

17.50-18.00 Anti-MAG neuropathy: role of IgM antibodies, the paranodal junction and juxtaparanodal potassium channels, **Nidhi Garg**, *Brain and Mind Centre, Sydney Medical School, The University of Sydney, NSW, Australia*

14.00-18.00 **► Charcot-Marie-Tooth & Related Neuropathies Consortium (CMTR)**
(*MARYLAND BALLROOM ABCD*)

14.00-14.30 Beyond Mendelian Genomics: The Expanding Concepts of Heredity In CMT
Stephan Zuchner
Chair: **Steven Scherer**

14.30-16.00 **Oral Platform Presentations**
Chairs: **Manisha Juneja** and **Stefano Previtali**
(Complete abstract information can be found in the abstract supplement)

14.30-14.45 Human Induced Pluripotent Stem Cell Differentiation towards a Schwann Cell Lineage
Robert Prior, *KU Leuven - Department of Neurosciences. VIB - Center for Brain and Disease Research*

14.45-15.00 A Kinase inhibitor improves neurofilament distribution in CMT2E human motor neuron axons, **Mario Saporta**, *University of Miami, Florida*



- 15.00-15.15** In Vivo Translational Profiling of Motor Neurons in Mouse Models of Charcot-Marie-Tooth Type2D, **Emily Spaulding**, *The Jackson Laboratory*
- 15.15-15.30** Predictive modeling reveals threonyl-tRNA synthetase (TARS) as a candidate gene for human axonal peripheral neuropathy, **Rebecca Meyer**, *University of Michigan*
- 15.30-15.45** Mutations in HSPB1 impair its mitochondrial role, **Elias Adriaenssens**, *Peripheral Neuropathy Research Group, Institute Born Bunge, University of Antwerp*
- 15.45-16.00** Unravelling Mechanisms of Axonal Loss in Late-onset Genetic Neuropathies, **Chiara Pisciotta**, *IRCCS Foundation, "C. Besta" Neurological Institute*
- 16.00-16.30** Coffee Break (MARYLAND FOYER)
- 16.30-17.00** Structural Variation Causing Inherited Neuropathies: A Paradigm for Genomic Organisation, Chromatin Interactions and Gene Dysregulation, **Marina Kennerson**, *ANZAC Research Institute, Australia*
Chair: **Charlotte Sumner**
- 17.00-17.45** **Oral Platform Presentations**
Chairs: **Jonathan Baets & Chiara Pisciotta**
(Complete abstract information can be found in the abstract supplement)
- 17.00-17.15** Mutations in Cell Adhesion Molecules Belonging to the CADM Family Cause Charcot-Marie-Tooth Disease, **Adriana Rebelo**, *University of Miami, Florida*
- 17.15-17.30** Expanding the phenotype of neuromuscular disorders: NGS reveals new genes responsible for recessive motor neuropathies, **Stefano Previtali**, *IRCCS San Raffaele Scientific Institute*
- 17.30-17.45** Alpha-spectrin haploinsufficiency through SPTAN1 nonsense mutations causes a spectrum of juvenile onset hereditary motor neuropathies, **Danique Beijer**, *Neurogenetics Group, CMN-VIB, University of Antwerp*
- 17.45-18.00** **Oral Poster Mini-Session**
Chairs: **Jonathan Baets & Chiara Pisciotta**
- 17.45-17.50** Autosomal recessive Charcot Marie Tooth disease: Clinical, Electrophysiology and genetic spectrum in a Tunisian series, **Emna Ellouz**, *Gabes Hospital*
- 17.50-17.55** Phenotypic and genotypic features of X-linked Charcot-Marie-Tooth type 1 and the study of pathogenic mechanism, **Xinghua Luan**, *Department of Neurology, Ruijin Hospital affiliated to Shanghai JiaoTong University*

PROGRAM – SUNDAY 22 JULY 2018

SUNDAY

17.55-18.00 Identification of Genetic Causes in Thai Charcot-Marie-Tooth Disease Children, **Oranee Sanmaneechai**, *Siriraj Hospital Mahidol University*

14.00-18.00 ► **Diabetic Neuropathy Consortium** (WATERTABLE BALLROOM)

14.00-14.15 Introduction to the Diabetic Neuropathy Consortium
Christopher Gibbons

14.15-16.00 **Keystone Target Talks**
Chairs: **Marta Campagnalo** and **James Russell**

14.15-14.35 Obesity Mechanisms as a Targetable Endpoint in Diabetic Neuropathy
Doug Wright

14.35-14.55 Keystone Target Talk - (Lipids and Neuropathy)
Mark Yorek

14.55-15.15 Nox5 as a therapeutic Target
Stephanie Eid

15.15-15.35 Stem Cells as a Potential Treatment in Diabetic Neuropathy
Nakamura

15.35-16.00 Muscarinic Receptors as a Targetable Endpoint in Diabetic Neuropathy
Paul Fernyhough

16.00-16.30 Coffee Break (MARYLAND FOYER)

16.30-17.00 Insulin Signaling Pathways as a Potential Therapeutic Target in DPN
Doug Zochodne

17.00-17.30 **Oral Platform Presentations**
Chairs: **Sherry Ho** and **Doug Wright**
(Complete abstract information can be found in the abstract supplement)

17.00-17.15 Metabolic Syndrome Components and Neurologic Outcomes in a Bariatric Surgery Population, **Brian Callaghan**, *University of Michigan*

17.15-17.30 Caloric Restriction in BKS Db/db Mice does not Ameliorate Diabetic Peripheral Neuropathy, **Phillipe O'Brien**, *University of Michigan*

17.30-18.00 **Oral Poster Presentations**
Chairs: **Stephanie Eid** and **Paul Fernyhough**
(Complete abstract information can be found in the abstract supplement)

17.30-17.35 Alterations in DRG Calcium Signaling and Mitochondrial Homeostasis in Painful Diabetic Neuropathy, **Sandra Hackelberg**, *Northwestern University*



- 17.35-17.40** Uncovering an Important Role for Monocarboxylate Transporter MCT1 in Diabetic Neuropathy, **Mithilesh Jha**, *Department of Neurology, Johns Hopkins University School of Medicine*
- 17.40-17.45** IGF-1 Signals Through Different Isoforms of AMPK to Control Mitochondrial Function in Adult Sensory Neurons, **Darrell Smith**, *St. Boniface Research Centre*
- 17.45-17.50** Induced expression of keratinocyte-derived BDNF by SIRT1 activation alleviates diabetic neuropathy, **Cheng-Ying Ho**, *University of Maryland School of Medicine*



- 18.00-19.00** **AKCEA Pharmaceuticals Symposium** (MARYLAND EF)
Hereditary Transthyretin (hATTR) Amyloidosis: An Underrecognized Cause of Peripheral Sensorimotor Neuropathy
Speakers: **Thomas Brannagan** and **Michelle Mauermann**
Chair: **Jim Dyck**

CSL Behring

Biotherapies for Life®

- 18.00-19.00** **CSL Behring Symposium** (MARYLAND ABCD)
- 18.00-18.05** Welcome and Introduction
Richard Lewis
- 18.05-18.20** Subcutaneous Immunoglobulin in CIDP: The Evidence
Thomas Harbo
- 18.20-18.35** Practical Aspects in Subcutaneous Immunoglobulin Administration
Melody Bullock
- 18.35-18.50** Objective Measures You Should Use to Monitor CIDP and Treatment Response
Jeffrey Allen
- 18.50-19.00** Q&A and Closing Remarks
Richard Lewis
- 19.00-20.00** **Junior Reception** (MARYLAND FOYER)
All are encouraged to attend.
Sponsored by:



PROGRAM – MONDAY 23 JULY 2018

- 07.30-18.00** Registration / Help Desk Open (MARYLAND FOYER)
- 07.30-08.30** Light Breakfast & Poster Viewing (BALTIMORE BALLROOM AB)
- 08.30-09.00** **Plenary Session: Arthur K Asbury Lecture** (MARYLAND BALLROOM ABCD)
Introduction by **Susumu Kusunoki**



Shared B Cell - Mediated Immunopathology in CIDP, myasthenia gravis, and other IgG4 - Mediated Diseases
Kevin O'Connor, Yale University, New Haven, CT, USA

- 09.00-10.30** **Platform Session II** (MARYLAND BALLROOM ABCD)
Chairs: **Kleopas Kleopa** and **Ruth Huizinga**
(Complete abstract information can be found in the abstract supplement)
- 09.00-09.15** Preliminary Phase 2 Results for ACE-083, Local Muscle Therapeutic, in Patients with CMT1 and CMTX, **Florian Thomas**, Hackensack University Medical Center and Seton Hall University
- 09.15-09.30** Allele-Specific RNA Interference: Precision Gene Therapy for Dominant Inherited Neuropathy, **Robert Burgess**, The Jackson Laboratory
- 09.30-09.45** Placebo Effect in The PATH Study of Subcutaneous Immunoglobulin in Chronic Inflammatory Demyelinating Polyneuropathy, **Richard Lewis**, Department of Neurology, Cedars-Sinai Medical Center
- 09.45-10.00** Atypical CIDP: diagnostic criteria, progression and response to therapy. Data from the Italian CIDP Database, **Pietro Doneddu**, Humanitas Research Hospital
- 10.00-10.15** Altered actin dynamics contributes to severe myelination defects following Schwann cell-specific deletion of phosphatidylinositol 4-kinase-IIIa, **Alejandro Alvarez-Prats**, National Institutes of Health (NIH/NICHD)
- 10.15-10.30** Regional Variation of Guillain-Barré Syndrome, **Alexandra Doets**, Erasmus University Medical Center
- 10.30-11.00** Coffee Break (MARYLAND FOYER)



11.00-11.30 **Plenary Session: PK Thomas Lecture** (MARYLAND BALLROOM ABCD)
Introduction by **Lucia Notterpek**



Phospholipid Metabolism and the Control of Schwann Cell Myelination

Alessandra Bolino, *San Raffaele Scientific Institute, Milan, Italy*

11.30-12.30 **Oral Poster Session II** (MARYLAND BALLROOM ABCD)

Chairs: **Claudia Sommer** and **Maureen Su**

(Complete abstract information can be found in the abstract supplement)

- 11.30-11.35** Mtmr2 loss impairs lysosome-associated intracellular signaling in Schwann cells: the impact on the myelin homeostasis, **Haesun Kim**, *Rutgers University*
- 11.35-11.40** Wnt Signaling in Schwann Cells Contributes to Peripheral Nerve Regeneration, **Benayahu Elbaz**, *The University of Chicago*
- 11:40-11:45** Mutations in Cell Adhesion Molecules Belonging to the CADM Family Cause Charcot-Marie-Tooth Disease, **Adriana Rebelo**, *University of Miami Miller School of Medicine*
- 11.45-11.50** The Importance of Genetic Screening of Voltage Gated Sodium Channels in Pure Small Fiber Neuropathy, **Amir Far**, *Maastricht University Medical Center*
- 11.50-11.55** CSF Sphingomyelin concentration: a myelin biomarker for acquired demyelinating neuropathies, **Lucilla Nobbio**, *University of Genoa*
- 11.55-12.00** Clinical course and outcome of Guillain-Barré syndrome in Bangladesh, **Badrul Islam**, *International Center for Diarrhoeal Disease Research, Bangladesh*
- 12.00-12.05** Immunoglobulin dosing in inflammatory neuropathy: An induction, maintenance and cessation algorithm, **Laura Compton**, *National Hospital for Neurology and Neurosurgery*
- 12.05-12.10** Serum neurofilament light chain in chronic inflammatory demyelinating polyneuropathy, **Gwen van Lieferloo**, *Academic Medical Center*
- 12.10-12.15** International Zika virus related Guillain-Barré Syndrome Outcome Study (IGOS-Zika): Update of a case-controlled study, **Sonja Leonhard**, *Department of Neurology, Erasmus Medical Centre*
- 12.15-12.20** Effectiveness of Limited plasma exchange (LPE) in Guillain-Barré syndrome (GBS), **Yan Lynn Aung**, *Yangon General Hospital*

PROGRAM – MONDAY 23 JULY 2018

- 12.30-13.00** **PNS Annual General Meeting** (MARYLAND BALLROOM ABCD)
All are welcome to attend
- 13.00-14.00** Lunch (BALTIMORE BALLROOM AB)
- 13.00-14.00** **Poster Session II** (BALTIMORE BALLROOM AB)
All Presenters are required to stand by posters from 13.00-14.00
- 14.00-18.00** **Special Interest Group (SIG) Parallel Sessions**
- 14.00-18.00** **► Inflammatory Neuropathy Consortium (INC)** (MARYLAND BALLROOM ABCD)
- 14.00-14.30** INC Business and Reviews of New Ongoing Projects
Chair: **Peter Van den Bergh**, *INC Vice-Chair*
- 14.00-14.10** IMAGiNe Study
Ingemar Merkies
- 14.10-14.20** CIDP Databases
Filip Eftimov
- 14.20-14.30** Italian CIDP Database: What we Have Learned from the First 500 Patients
Eduardo Nobile-Orazio
- 14.30-15.00** Invited Talk: Paraneoplastic Neuropathies
JC Antoine
Chair: **Emilian Delmont**
- 15.00-15.50** **Oral Platform Presentations**
Chairs: **Islam Badrul** and **Joyce Roodbol**
(Complete abstract information can be found in the abstract supplement)
- 15.00-15.10** Second IVIg course in Guillain-Barré syndrome with poor prognosis: the non-randomized I-SID study, **Christine Verboon**, *Erasmus MC*
- 15.10-15.20** The 2016 Singapore ZIKV Outbreak did not cause a Surge in Guillain-Barré Syndrome, **Brandon C.J. Ng**, *Yong Loo Lin School of Medicine, National University of Singapore, Singapore*
- 15.20-15.30** The Natural History of POEMS Syndrome: practice review from the largest UK single centre experience, **Stephen Keddie**, *National Hospital of Neurology and Neurosurgery*
- 15.30-15.40** Prevalence and Associations of Peripheral Neuropathy at Disease-Onset in ANCA-Associated Vasculitides: Data from DCVAS Study, **Michael Collins**, *Medical College of Wisconsin*
- 15.40-15.50** Guillain-Barré Syndrome in Denmark-a Population Based Study of Epidemiology and Clinical Characteristics, **Helle Al-Hakem**, *Department of Neurology, Aarhus University Hospital*



- 16.00-16.30** Coffee Break (MARYLAND FOYER)
- 16.30-17.00** Debates: Perspectives on Electrodiagnostic Criteria for Guillain-Barre Syndrome
Nortina Shahrizalia and **Peter Van den Bergh**
Chair: **Umpathi Thiruganam**
- 17.00-17.30** Invited Talk: Updated on Multifocal Motor Neuropathy
Ludo van der Pol
Chair: **Robert Hadden**
- 17.30-18.00** **Oral Platform Presentations**
Chair: **Govind Chavada**
(Complete abstract information can be found in the abstract supplement)
- 17.30-17.40** Axial Sensory Loss in Acquired Demyelinating Polyneuropathies, **João Paulo Alves**, *Ribeirão Preto Medical School, University of São Paulo*
- 17.40-17.50** Clinical and electrodiagnostic findings in neuropathy with anti-Fibroblast Growth Factor Receptor 3 antibodies, **Raghav Govindarajan**
- 17.50-18.00** A Three-year Nerve Ultrasound Longitudinal Study in 19 Patients with CIDP, **Laura Fionda**, *Sant'Andrea Hospital, Sapienza University of Rome*
- 14.00-18.00** ▶ **Charcot-Marie-Tooth & Related Neuropathies Consortium**
(MARYLAND BALLROOM EF)
- 14.00-14.30** PMP22 Antisense Oligonucleotides in Rodent Models of the Charcot-Marie-Tooth Disease Type 1A Duplication
John Svaren
Chair: **Michael Shy**
- 14.30-16.00** **Oral Platform Session**
Chairs: **Andoni Echaniz-Laguna** and **Kayla Cornett**
(Complete abstract information can be found in the abstract supplement)
- 14.30-14.45** CMT2, CRISPR and Stem Cells: Disease Mechanism and Genome Surgery, **Bruce Conklin**, *Gladstone Institutes*
- 14.45-15.00** Status of the Pivotal Phase III Study of PXT3003 for Charcot-Marie-Tooth Type 1A disease (CMT1A), **Rene Goedkoop**, *Pharnext SA*
- 15.00-15.15** 1st in Human IT Gene Transfer for GAN:Review of Safety, Immunology & Interim Analysis of Efficacy, **Dimah Saade**, *NIH, NINDS, NNDCS*
- 15.15-15.30** Therapeutic effects of HDAC6 inhibitors in models of inherited and acquired neuropathies, **Ludo Van Den Bosch**, *KU Leuven & VIB*
- 15.30-15.45** Development and validation of the CMT Infant Scale, **Melissa Mandarakas**, *University of Sydney; Sydney Children's Hospitals Network (Randwick and Westmead)*

PROGRAM – MONDAY 23 JULY 2018

- 15.45-16.00** Serum biomarker discovery for Charcot-Marie-Tooth disease, **Matthew Jennings**, *Newcastle University*
- 16.00-16.30** Coffee Break (MARYLAND FOYER)
- 16.30-17.00** Overview of Lesson Learnt from Novel Therapies for Hereditary ATTR-neuropathy, **Teresa Coelho**
Chair: **Mary Reilly**
- 17.00-18.00** **Platform Session**
Chairs: **Wilson Marques** and **Andrea Cortese**
(Complete abstract information can be found in the abstract supplement)
- 17.00-17.15** Evolution of Amyloid Fibrils in Transthyretin Familial Amyloid Polyneuropathy: an Ultrastructural Study, **Haruki Koike**, *Nagoya University Graduate School of Medicine*
- 17.15-17.30** Development and Validation of the Transthyretin Familial Amyloid Polyneuropathy Score (TTR-FAP Score), **Teresa Coelho**, *Unidade Corino de Andrade, Centro Hospitalar do Porto*
- 17.30-17.45** Identifying the transition from TTR carrier to TTR neuropathy patient, **Michael Polydefkis**, *Johns Hopkins Hospital*
- 17.45-18.00** Familial Amyloid Polyneuropathy: Impact of Biopsies and Mutations on Diagnostic Considerations, **Christopher Gibbons**, *Beth Israel Deaconess Medical Center, Harvard Medical School*
- 14.00-18.00** ➤ **Diabetic Neuropathy Consortium** (WATERTABLE BALLROOM)
- 14.00-14.30** Moderated Panel Debate Neurophysiology endpoint talks (EMG, QST, Autonomic)
Vera Bril, James Russell, Amanda Peltier
Chair: **Eva Feldman**
- 14.30-15.00** Moderated Panel Debate: Structural Endpoint Talks (CCM, Skin Biopsy, Sural Nerve Biopsy)
Rayaz Malik, Michael Polydefkis and Gordon Smith
Chair: **James Russel**
- 15.00-15.20** Perspective on Endpoints
Eva Feldman and Roy Freeman
Introduction by **Rodica Pop-Busui**
- 15.20-15.40** What's New in DLRPN
Jim Dyck
Introduction by **Rodica Pop-Busui**

MONDAY

- 15.40-16.00** What's New in TIND
Christopher Gibbons
 Introduction by **Rodica Pop-Busui**
- 16.00-16.30** Coffee Break (MARYLAND FOYER)
- 16.30-17.00** Diabetic Peripheral Neuropathy and the Opioid Crisis
Brian Callaghan
 Introduction by **Lindsay Zilliox**
- 17.00-17.30** **Platform Session**
 (Complete abstract information can be found in the abstract supplement)
- 17.00-17.15** A Randomized, Blinded, Lifestyle Intervention Study Improves the Expiration:Inspiration Ratio in Diabetic Neuropathy, **Lindsay Zilliox**, *University of Maryland School of Medicine*
- 17.15-17.30** Sensory Axons Inhibit Motor Axon Regeneration in vitro, **Thomas Brushart**, *Johns Hopkins*
- 17.30-18.00** **Oral Poster Session**
 Chairs: **Phillipe O'Brien** and **Amanda Peltier**
 (Complete abstract information can be found in the abstract supplement)
- 17.30-17.35** Expression of GAP-43 in Sensory Axon Terminals, **Xin Pan**, *Department of Neurology, Johns Hopkins School of Medicine*
- 17.35-17.40** Risk Of Developing Treatment-Induced Neuropathy In Diabetes; A Nested Case-control Study, **Jing Hang Randy Soh**, *Yong Loo Lin School of Medicine, National University Singapore*
- 17.40-17.45** An immortalized human DRG neuronal cell line to model diabetic and chemotherapy induced peripheral neuropathies, **Weiran Chen**, *Department of Neurology, The Johns Hopkins Medical School*
- 17.45-17.50** Topiramate as a Disease Modifying Therapy for Cryptogenic Sensory Peripheral Neuropathy: Design and Analysis Plan, **A. Gordon Smith**, *Virginia Commonwealth University*



- 18.00-19.00 Alnylam Pharmaceuticals Symposium** (MARYLAND BALLROOM EF)
A History of hATTR Amyloidosis: Understanding the Disease and Evolution of Diagnostic Strategies
Chair: **Michael Polydefkis**
Michael Slay and **Brian Drachman**

GRIFOLS

- 18.00-19.00 Grifols Symposium** (MARYLAND BALLROOM ABCD)
Small Fiber Polyneuropathy and Autoimmunity: Preparing For Clinical Trials
Chair: **Anne Louise Oaklander**
- 18.00-18.05** SPIN Award ceremony
- 18.05-18.15** Overview of apparently autoimmune small fiber polyneuropathy, **Anne Louise Oaklander** (Boston, USA)
- 18.15-18.35** Intravenous immunoglobulin therapy for small fiber neuropathy: protocol and study update, **Ingemar Merkies** (Maastricht, The Netherlands)
- 18.35-18.55** Inflammatory markers in treatment induced neuropathy of diabetes: Protocol and study update, **Christopher Gibbons** (Boston, USA)
- 18.55-19.00** A double-blind, placebo controlled trial of IVIG in patients with small fiber neuropathy associated with autoantibodies to TS-HDS and FGFR3: protocol and study update, **Christopher Gibbons** (Boston, USA)
- 19.00-20.00 All Member Reception** (MARYLAND FOYER)
All members are encouraged to attend the reception.



07.30-18.00 Registration / Help Desk Open (MARYLAND FOYER)

07.30-08.30 Light Breakfast & Poster Viewing (BALTIMORE BALLROOM AB)

08.30-09.00 **Plenary Session: Jack Griffin Lecture** (MARYLAND BALLROOM ABCD)
Introduction by **Paul Fernyhough**



Regeneration

David Parkinson, *Plymouth University School of Medicine and Dentistry, Plymouth, United Kingdom*

09.00-10.30 **Platform Session III** (MARYLAND BALLROOM ABCD)

Chairs: **Jim Dyck** and **Brett Morrison**

(Complete abstract information can be found in the abstract supplement)

09.00-09.15 Trapped in the epineurium: Early entry into endoneurium is restricted to neurotogenic T-cells in EAN, **Anne Mausberg**, *University Hospital Essen, Department of Neurology*

09.15-09.30 Impaired motor axon radial sorting and growth precede an early wave of degeneration in SMA, **Lingling Kong**, *Johns Hopkins University*

09.30-09.45 A promising new drug candidate for the treatment of anti-MAG neuropathy, **Pascal Haenggi**, *Institute of Molecular Pharmacy, University of Basel*

09.45-10.00 Two-Photon Imaging of Human Neuromuscular Junction Degradation After Traumatic Peripheral Nerve Injury, **Ranjan Gupta**, *University of California, Irvine*

10.00-10.15 Autoimmune CRMP5 neuropathy phenotype and outcome defined from 105 cases, **Divyanshu Dubey**, *Mayo Clinic*

10.15-10.30 A randomized controlled trial with Rituximab in patients with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP), **Eduardo Nobile-Orazio**, *IRCCS Istituto Clinico Humanitas, Milan University*

10.30-11.00 Coffee Break (MARYLAND FOYER)

PROGRAM – TUESDAY 24 JULY 2018

11.00-11.30 Plenary Session: Pembroke Lecture (MARYLAND BALLROOM ABCD)
Introduction by **Marina Kennerson**



Cell Biology Lecture
Craig Blackstone, *NINDS Cell Biology Section, Bethesda, MD, USA*

11.30-12.30 Oral Poster Session III (MARYLAND BALLROOM ABCD)
Chairs: **Anthony Antonellis** and **Matilde Laura**
(Complete abstract information can be found in the abstract supplement)

- 11.30-11.35** PACSIN-1 released from injured axons may activate the Schwann Cell Repair Program after PNS injury, **Curtis Triebswetter**, *University of California, San Diego*
- 11.35-11.40** Molecular phenotyping of neurons derived from CMT2 patient-iPSC lines, **Manisha Juneja**, *University of Antwerp*
- 11.40-11.45** Focal Reduction of Monocarboxylate Transporter 1 (MCT1) in Macrophages Delays Peripheral Nerve Regeneration, **Brett Morrison**, *Johns Hopkins University*
- 11.45-11.50** Digit Wrinkle Scan©: from Normative Values to its Clinical Applicability in Small Fiber Neuropathy, **Isis Joosten**, *Maastricht University Medical Center*
- 11.50-11.55** Temporal variability of the binding specificity of IgM M-proteins in anti-MAG neuropathy, **Susumu Kusunoki**, *Kindai University Faculty of Medicine*
- 11.55-12.00** Ultrastructural Mechanisms of Macrophage-induced Demyelination in Chronic Inflammatory Demyelinating Polyneuropathy, **Haruki Koike**, *Nagoya University Graduate School of Medicine*
- 12.00-12.05** Clinical Heterogeneity of Paraproteinemic Polyneuropathies, **Aleksa Palibrk**, *Neurology Clinic, Clinical Centre of Serbia*
- 12.05-12.10** $\alpha 7$ nicotinic acetylcholine receptors (nAChRs) play an important role in chemotherapy-Induced Peripheral Neuropathy in mice, **M. Imad Damaj**, *Virginia Commonwealth University*
- 12.10-12.15** Schwann Cell-derived Desert Hedgehog Regulates the Endoneurial Fibroblast Phenotype in Peripheral Nerves via Gli1, **Brendan Zotter**, *NYU School of Medicine*
- 12.15-12.20** Practical Application of Subcutaneous Immunoglobulin For Maintenance Treatment In CIDP: The PATH Study, **Mazen Dimachkie**, *University of Kansas Medical Center*



- 12.30-13.00** Lunch & Poster Viewing (BALTIMORE BALLROOM AB)
- 13.00-14.00** **Poster Session III** (BALTIMORE BALLROOM AB)
All Presenters to stand by posters from 13.00-14.00.
- 14.00-16.00** **Clinical Trials Updates** (MARYLAND BALLROOM ABCD)
Chairs: **Mary Reilly & David Cornblath**
- 14.00-14.10** Long-term Safety and Efficacy of Subcutaneous Immunoglobulin IgPro20 in CIDP: the PATH Extension Study
Ivo Van Schaik
- 14.10-14.20** Benefit-Risk Profile of Intravenous Immunoglobulin (IVIg) and Subcutaneous Immunoglobulin (SCIg) in CIDP: the PATH Study
Amgad Shebl
- 14.20-14.30** Clinical Characteristics of Patients with Active Disease: Can we Optimise Patient Selection for Trials
Mahima Kapoor
- 14.30-14.40** OPTIC Trial: Intravenous Immunoglobulin And Intravenous Methylprednisolone As Induction Treatment In CIDP (protocol)
Sander Bus
- 14.40-14.50** Pilot Screening Study of B Cell Depletion Therapy in CIDP
Mazen Dimachkie
- 14.50-15.00** Japanese eculizumab trial for Guillain-Barré syndrome (JET-GBS): The current status and perspective
Sonoko Misawa
- 15.00-15.10** Small volume plasma exchange for Guillain-Barré syndrome: a phase II safety and feasibility study
Badrul Islam
- 15.10-15.20** Second IVIg Course in Guillain-Barre syndrome patients with poor prognosis (SID-GBS trial)
Christa Walgaard
- 15.20-15.30** Patient Assisted Intervention for Neuropathy: Comparison of treatment in real life situations (PAIN-CONTRoLS)
Mamatha Pasnoor
- 15.30-15.40** Late Breaking Abstract: A Novel Recessive Pentanucleotide Repeat Expansion is a Frequent Cause of Late-onset Sensory Ataxic Neuropathy, **Andrea Cortese**, MRC Centre for Neuromuscular Diseases, UCL Institute of Neurology

PROGRAM – TUESDAY 24 JULY 2018

16.00-16.30 Coffee Break (MARYLAND FOYER)

16.30-17.30 **Closing Keynote Lecture** (MARYLAND ABCD)
Introduction by **Charlotte Sumner**



Spinal Muscular Atrophy from Gene to Treatment
Arthur Burghes, *The Ohio State University College of Medicine, Columbus, OH, USA*

17.30-18.00 **Awards & Prizes** (MARYLAND ABCD)

18.30 **Closing Reception at Camden Yards:** Orioles vs Red Sox Baseball Game
Buses will depart from the main hotel entrance. Please make sure to bring your badge with you in order to get into the game.





09.00-11.00 **SPECIAL INTEREST GROUP (SIG): Toxic Neuropathy** (MARYLAND EF)
Hosted by **Guido Cavaletti**



Table 1



Akcea Therapeutics

www.akceatx.com
info@akceatx.com

Akcea Therapeutics is a development and commercialization company focused on helping patients with serious and rare diseases. The name “Akcea” is derived from the Greek word for value and worth. Something that has “akcea” is not common, but precious and rare. Our name supports the value we are creating for the healthcare provider community, patients, and their families. We are driven by knowing that patients depend on us.

Table 2



CSL Behring
www.cslbehring.com

CSL Behring is a global biotherapeutics leader driven by our promise to save lives. We meet patients’ need using the latest technologies to develop and deliver innovative biotherapies that are used to treat serious and rare conditions such as coagulation disorders, primary immune deficiencies, hereditary angioedema and inherited respiratory disease.

Table 3



Anylam Pharmaceuticals, Inc.

www.alnylam.com
info@alnylam.com

Anylam is leading the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to transform the lives of people with rare genetic, cardio-metabolic, and hepatic infectious diseases with unmet need. Anylam’s investigational medicines include four late-stage product candidates, with one under regulatory review.

Table 4



GRIFOLS

www.grifols.com
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.

Table 5 GBS/CIDP Foundation International



www.gbs-cidp.org
lori.basiege@gbs-cidp.org

The GBS|CIDP Foundation International is the preeminent global nonprofit supporting individuals and their families affected by GBS, CIDP and related conditions. Through providing support, education, research and advocacy, the Foundation works for a future where no one suffers alone and everyone has access to the right diagnosis and the right treatment, right away.

EXHIBITOR DIRECTORY

Table 6



CMTA

www.cmtausa.org
info@cmtausa.org

The Charcot-Marie-Tooth Association is the world's leading nonprofit organization dedicated to finding a cure for CMT. The CMTA's Strategy to Accelerate Research (STAR) program brings top researchers together with pharmaceutical partners so that scientific breakthroughs are possible. The CMTA also offers community services including 70 branches, a camp and conferences.

Table 9



Amyloid Support Groups (ASG)

www.amyloidosisupport.org
info@amyloidosisupport.org

Amyloidosis Support Groups (ASG) is a 501 (C) 3 Non Profit founded in 2004. We run face to face support groups in more than 25 cities. We also have 8 Support Groups on Facebook and two online List-Servs. We published a 10 minute animated video and educational booklet, both of which can be found at www.amyloidaware.com.

Table 7 Hereditary Neuropathy Foundation



www.hnf-cure.org
info@hnf-cure.org

Hereditary Neuropathy Foundation (HNF) is a non-profit 501(c)3 organization which mission is to increase awareness and accurate diagnosis of Charcot-Marie-Tooth (CMT) and related INHERITED NEUROPATHIES, support patients and families with critical information to improve quality of life, and fund research that will lead to treatments and cures.

Table 10



AFANP

www.alliance-neuro.org

Table 14

Shire Commercial

www.shire.com



Shire is the global leader in serving patients with rare diseases. We strive to develop best-in-class therapies across a core of rare disease areas, supplemented by diversified capabilities in highly specialized conditions. We feel a strong sense of urgency to address the high unmet medical needs of these patient communities.

Table 8



The Foundation for Peripheral Neuropathy

www.foundationforpn.org
info@tffpn.org

The Foundation for Peripheral Neuropathy (FPN) is a public charity foundation whose ultimate goal is to utilize every means and opportunity to dramatically improve the lives of those living with this painful and debilitating disorder. We achieve this mission by serving as the premier resource of information for patients, funding collaborative efforts of leading scientists, raising awareness, and accelerating a cure for peripheral neuropathy.

Table 15

Terumo BCT, Inc.

www.terumobct.com
NAConferences@terumobct.com



Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We are committed to supporting clinicians and patients with education and research support for plasma exchange. Learn more about the immunomodulatory effects of plasma exchange in reducing disease mediators. In addition, learn about the convenience and safety of using plasma exchange in an outpatient setting using peripheral venous access. Contact us for more information: NAConferences@terumobct.com

Table 17

Invitae

www.invitae.com



Invitae's mission is to bring comprehensive genetic information into mainstream medical practice to improve the quality of healthcare for billions of people. Our goal is to aggregate most of the world's genetic tests into a single service with higher quality, faster turnaround time and lower prices. Visit www.invitae.com.

Table 18

Mayo Medical Laboratories

www.mayomedicallaboratories.com



Mayo Medical Laboratories (MML) is a global reference laboratory operating within Mayo Clinic's Department of Laboratory Medicine and Pathology. MML supports neurology practices through comprehensive testing services with specialization in autoimmune, biochemical, and genetic testing. MML also offers muscle and nerve pathology services. Learn more at MayoMedicalLaboratories.com/neurology.

Table 19

Shire Medical

www.shire.com/en



Shire is the global leader in serving patients with rare diseases. We strive to develop best-in-class therapies across a core of rare disease areas, supplemented by diversified capabilities in highly specialized conditions. We feel a strong sense of urgency to address the high unmet medical needs of these patient communities.

Table 20

Biogen

www.biogen.com



Table 21

BÜHLMANN Laboratories AG

www.buhlmannlabs.com
sas@buhlmannlabs.com



BÜHLMANN Diagnostics Corp (BDC), the North American affiliate of BÜHLMANN Laboratories AG, offers a comprehensive neuroimmunology product line comprised of robust assays for simple, esoteric testing for important neuroimmune markers and it is supported by numerous clinical publications. Assays include BÜHLMANN GanglioCombi™ MAG ELISA, Anti-MAG Autoantibodies ELISA, and Anti-SGPG Autoantibodies ELISA.

EXHIBITOR DIRECTORY

Table 22

Disarm Therapeutics

www.disarmtx.com
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.

Table 23

Vertex Pharmaceuticals

www.vrtx.com



Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases. Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. Vertex is consistently recognized as one of the industry's top places to work, including being named to Science magazine's Top Employers in the life sciences ranking for eight years in a row.

Table 24

Acceleron Pharma, Inc.

www.acceleronpharma.com
info@acceleronpharma.com



Acceleron is a clinical-stage biopharmaceutical company dedicated to discovery, development, and commercialization of therapeutics to treat serious and rare diseases. Acceleron's leadership in TGF-beta biology and protein engineering generates innovative compounds that help regulate cellular growth and repair. Acceleron is advancing two distinct Myostatin+ agents, ACE-083 and ACE-2494, in neuromuscular diseases.

Table 25

Alnylam Pharmaceuticals, Inc.

www.alnylam.com
info@alnylam.com



Alnylam is leading the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to transform the lives of people with rare genetic, cardio-metabolic, and hepatic infectious diseases with unmet need. Alnylam's investigational medicines include four late-stage product candidates, with one under regulatory review.

Table 26

Terumo BCT, Inc.

www.terumobct.com
NAConferences@terumobct.com



Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We are committed to supporting clinicians and patients with education and research support for plasma exchange. Learn more about the immunomodulatory effects of plasma exchange in reducing disease mediators. In addition, learn about the convenience and safety of using plasma exchange in an outpatient setting using peripheral venous access. Contact us for more information: NAConferences@terumobct.com.

Table 27**Octapharma**www.octapharma.com

Headquartered in Lachen, Switzerland, Octapharma is one of the largest human protein manufacturers in the world, developing and producing human proteins from human plasma and human cell lines. As a family-owned company, Octapharma believes in investing to make a difference in people's lives and has been doing so since 1983; because it's in our blood.

Octapharma employs more than 7,100 people worldwide to support the treatment of patients in 113 countries with products across three therapeutic areas: Immunotherapy, Haematology and Critical care.

Table 29**Kedrion Biopharma**[www.kedrion.us/
r.mccune@kedrion.com](http://www.kedrion.us/r.mccune@kedrion.com)

Kedrion Biopharma, an international biopharmaceutical company with corporate headquarters in Tuscany, Italy, specializes in the production of plasma-derived therapies for rare diseases and conditions such as hemophilia and primary immunodeficiency. Kedrion Biopharma's US headquarters are in Fort Lee, New Jersey with a global presence in about 100 countries.

Table 28**Pharnext**[www.pharnext.com/en/
contact@pharnext.com](http://www.pharnext.com/en/contact@pharnext.com)

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases that lack curative and/or disease-modifying treatments. Pharnext has developed a new drug discovery paradigm based on big genomic data and artificial intelligence: PLEOTHERAPY™. PXT3003, Pharnext's lead product, is currently in an international Phase 3 trial for the treatment of Charcot-Marie-Tooth disease type 1A, results are expected in the second half of 2018.



Hereditary Transthyretin (hATTR) Amyloidosis: An Underrecognized Cause of Peripheral Sensorimotor Neuropathy

OBJECTIVES

- To increase awareness of hATTR amyloidosis among neurologists
- To describe the multisystemic clinical presentation of hATTR amyloidosis and highlight diagnostic challenges
- To improve recognition of hATTR amyloidosis among neurologists

FACULTY

- P. James B. Dyck, MD (Chair)**
Mayo Clinic, Rochester, Minnesota
- Thomas H. Brannagan III, MD**
Columbia University, New York, New York
- Michelle L. Mauermann, MD**
Mayo Clinic, Rochester, Minnesota

OVERVIEW

Please join P. James B. Dyck, MD, and our expert faculty for a one-hour symposium that will provide an overview of hATTR amyloidosis, an underrecognized and often overlooked cause of peripheral sensorimotor neuropathy. The multisystemic manifestations and substantial overlap in clinical presentation of hATTR amyloidosis with other more common conditions make diagnosis challenging. In this symposium, the clinical features, variable presentation, and common challenges encountered with the diagnosis of hATTR amyloidosis will be reviewed. A retrospective look at a patient case study will be used throughout each presentation to exemplify key concepts that facilitate timely and accurate diagnosis, ultimately leading to improved patient outcomes.

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Sunday, July 22, 2018
6:00–7:00 PM**

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Octapharma is one of the largest human plasma protein products manufacturers in the world and has been committed to patient care and medical innovation since 1983. Our core business is the development and production of human plasma proteins from human plasma and human cell lines. Octapharma employs more than 7,600 people worldwide to support the treatment of patients in 113 countries with coagulation disorders, immune disorders, and other critical illnesses.

THE POTENTIAL TO TRANSFORM

— the treatment of —
hATTR amyloidosis

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- A newly approved Ig therapy for CIDP
- One immunoglobulin portfolio, two impactful therapies, thousands of patients treated¹

Reference: 1. Data on file. Available from CSL Behring as DOF HIZ-005.

CIDP = chronic inflammatory demyelinating polyneuropathy

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