

ASENT 2024 ANNUAL MEETING



GLOBAL NEUROTHERAPEUTICS
CONFERENCE
MARCH 12 - 14, 2024

HYATT REGENCY BETHESDA,
BETHESDA, MD



ADVANCE PROGRAM



Andrew J. Cole, M.D., F.R.C.P.(C.)
Professor of Neurology
Harvard Medical School
Director, MGH Epilepsy Service
Chief, Division of Clinical
Neurophysiology
Vice-Chair for External Affairs,
Dept. of Neurology
President, ASENT

Dear Colleagues,

After three years of very successful virtual Annual Meetings, we are thrilled to meet in person in 2024 in Bethesda. The ASENT2024 Annual Meeting will highlight the latest therapeutic advances in a broad range neurologic disease states, processes and delivery methods, including nine scientific symposia, a scientific debate, and a Covid update. A few highlights include:

On Thursday, March 14, 2024, the plenary session “Pre-clinical to First in Human in Drug Development” will feature Nathan Fountain, M.D., Thomas E. Worrell, Jr, Professor of Neurology and Epileptology at University of Virginia and Jim Doherty, Ph.D., of Sage Therapeutics to compare the similarities and differences in the preclinical process and infrastructure when developing a neurotherapeutic. As ASENT President, I will personally moderate this fireside chat style discussion.

The two additional unopposed plenary sessions will take place: “Fluid Biomarkers as Endpoints for Neurodegenerative Diseases” on Tuesday, March 12, 2024, and “Parkinson's Disease Biological Staging and Implications for Therapeutic Trials” on Wednesday, March 13, 2024. Other unopposed sessions include “NIH Update on COVID and CNS disease” with speaker Avindra Nath, M.D., Senior Investigator, NINDS, and a Beta Amyloid Debate

will be held Wednesday, March 13, 2024.

Attendees will also have an opportunity to hear top abstracts selected for presentation by the Abstract Review Committee. On Wednesday, March 13, 2024, as many as 20 selected abstracts will be presented as part of the ASENT Pipeline Datablitz. Abstracts qualify for an ASENT Pipeline Data Blitz presentation when they are submitted as an Abstract Submission. Abstracts can be submitted via <https://asent.org/AnnualMeeting2024>

Other special events include the Careers in Neuroscience Dinner, during which invited speakers showcase the wide range of career opportunities available in neuroscience related fields. Each presenter will take a few minutes to share an overview of their career and highlight any unique career opportunities they have discovered along the way. Participants and faculty from the Training in Neurotherapeutics Discovery and Development for Academic Scientists course also participate in this dynamic event. This reception and dinner are a wonderful way to kick off the meeting on Tuesday, March 12, 2024.

We are excited that the inaugural ASENT Training in Clinical Trials course will be held on Monday, March 12 - Tuesday, March 13, 2024, co-located with the Annual Meeting. Developed by ASENT leadership and led by outstanding faculty, this 1 ½ day course will introduce researchers and clinicians to the principles of neurotherapeutic clinical trial development and implementation.

Finally, ASENT continues its dedication to our international colleagues. We are pleased to welcome guests from the Japanese Society of Neurological Therapeutics and are honored to continue the tradition of neurological education and research among academic neurologists around the world. I, along with the Board of Directors and the Program Committee, invite you to make the most of this outstanding meeting.

Best,
Andrew J. Cole, M.D., F.R.C.P.(C.)
Harvard Medical School
President, ASENT

WHY ATTEND ASENT2024

ASENT2024 is the premier neurotherapeutics conference where senior leaders through junior faculty and trainees from across payers, providers, employers, investors, fast-growing startups, industry, academia, policymakers, funders and innovation centers gather to ask one question: how can we improve the process of the development and discovery of neurotherapeutics?

Attendees are often surprised to find that more than 50% of participants are practicing physicians and the caliber of speakers is outstanding for a conference that provides ample opportunity to connect, network and converse. Additionally the scientific content showcases the leading edge in neurotherapeutics including innovations across disease states, novel delivery systems, gene therapy, the latest drug therapies and devices. The event features plenary sessions, fireside chats, panel discussions, business roundtables, and networking meetings.

MEETING DETAILS

ASENT 2024 Meeting Dates

Tues, March 12 - Thurs, March 14, 2024

Registration Deadline

March 12, 2024

asent.org/annualmeeting2024

Hotel Reservation Deadline

February 10, 2024

Meeting Location

Hyatt Regency Bethesda
Metro Center, 1,
Bethesda, MD 20814

asent.org/annualmeeting2024

WHO ATTENDS

Clinician-

Investigators

Neuroscience

Trainees

Neuroscientist

Researchers

Industry

Research

Scientists

Clinicians

Advocacy Group

Leaders

Chairs of

Neurology

Chief Medical

Officers

Chief Scientific

Officers

CEOs

Founders

Funders

Investors

Payers

Innovation

Centers

CROs

Journal Editors

Health

Journalists

Drug and Device

Companies

Communication

Companies

Health Systems

FDA

NIH

All ARE

WELCOME!

50% ARE

Prescribers

WHAT IS ASENT?

The **American Society for Experimental Neurotherapeutics (ASENT)** is an independent non-profit organization established in 1997 by leaders in academia, government, advocacy and industry to facilitate the process by which new therapies are made available to patients with neurological disorders. Its primary goal is to encourage and advance the development of improved therapies for diseases and disorders of the nervous system.

CONFERENCE INFORMATION AT A GLANCE

CONFERENCE DATES

March 12 - 14, 2024

PROGRAM

Registered sponsors are encouraged to attend all conference sessions, meals, receptions & events.

LOCATION

Hyatt Regency Bethesda
Bethesda, MD

REGISTRATION

Registration is open & is complimentary in most sponsorship packages.

HOUSING

Discounted housing is available at the Hyatt Regency Bethesda. Rates begin at \$219. Housing can be booked here: asent.org/annualmeeting2024

ABSTRACT SUBMISSION DATES

Opens: October 1, 2023

Closes: February 1, 2024

Submit your abstract here: asent.org/annualmeeting2024

PIPELINE SUBMISSION DATES

Opens: October 1, 2023

Closes: February 1, 2024

Submit your abstract here: asent.org/annualmeeting2024

OTHER ACTIVITIES

On March 11-12, The inaugural ASENT Clinical Trials Course will take place onsite. The Neurotherapeutics Drug Discovery and Development Course will run onsite March 12-15 as well.

REGISTRATION RATES

	MEMBER RATES	NON MEMBER RATES
AAN Member	\$250	\$375
Academic Member	\$275	\$475
Advocacy Member	\$175	\$250
Cinician Member	\$275	\$475
Government Member	\$275	\$325
Industry Member	\$425	\$675
JSNT Member	\$200	\$275
LMIC Member	\$150	\$200
Retired Member	\$250	\$375
Trainee Member	\$175	\$250

Register at asent.org/annualmeeting2024

MEETING OVERVIEW

ASENT2024 | ADVANCE PROGRAM

MONDAY, MARCH 11, 2024

7:00am - 5:30pm <i>Regency III</i>	ASENT Clinical Trials Course (APPLICATION ONLY)
5:30pm - 8:00pm <i>Regency I</i>	Reception and Dinner with Board and Program Committee and JSNT Delegates

TUESDAY, MARCH 12, 2024

7:30am - 12:00pm <i>Regency III</i>	ASENT Clinical Trials Course (APPLICATION ONLY)
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TUESDAY, MARCH 12, 2024

7:00am - 8:00am <i>Regency Ballroom Foyer</i>	Registration and Breakfast
8:00am - 8:15am <i>Regency I</i>	Presidential Welcome Remarks
8:15am - 10:15am <i>Regency I</i>	<p>Symposium: Fluid Biomarkers as endpoints for neurodegenerative diseases</p> <p>This symposium is offered in partnership with the Japanese Society of Neurological Therapeutics. We are honored to have a long partnership with JSNT and pleased to present their delegate.</p> <p>DESCRIPTION: Fluid Biomarkers are being utilized to study neurodegenerative diseases, including Alzheimers and Parkinsons Disease.</p> <p>CHAIR: Larry Altstiel, MD, PhD, ProMIS Neurosciences COCHAIR: Gail Farfel, PhD, Consultant</p> <p>FACULTY Dr. Ayami Okuzumi, Juntendo University School of Medicine, JSNT Presenter Robert Bowser, PhD Barrow Neurological Institute</p>
10:15am - 10:30am <i>Regency Ballroom Foyer</i>	Networking Break
10:30am - 12:00pm <i>Regency I</i>	<p>Symposium: Platform Clinical Trials in Neurology</p> <p>DESCRIPTION: The session will focus on how neurodegenerative disorders can benefit from platform designs using The HEALEY ALS Platform Trial as an example to showcase how the collaborative effort and enrollment in the trial can benefit neurotherapeutics trials.</p> <p>CHAIR: Merit Cudkowicz, MD, MSc, Harvard Medical School/MGH COCHAIR: Amir Tamiz, PhD, NINDS</p> <p>PRESENTATIONS: The NINDS and Master Protocols for Platform Trials: Many Neurological Needs, So Little Time Clinton Wright, MD, MS, NINDS</p> <p>Healey ALS Platform Trials: Lessons and Future Merit Cudkowicz, MD, MSc, Harvard Medical School/MGH</p> <p>Statistical Science of Platform Trials: The Balance of Flexibility and Inference Melanie Quintana, PhD, Berry Consultants</p> <p>PANEL DISCUSSION INCLUDING: Irfan Qureshi, MD, CMO, Biohaven Teresa Buracchio, MD, CDER, FDA</p>

TUESDAY, MARCH 12, 2024

12:00pm - 1:30pm <i>Regency Ballroom Foyer</i>	Lunch Break
12:30pm - 1:30pm <i>TBD</i>	Program Committee and Board Meeting
1:30pm - 3:00pm <i>Regency IV</i>	<p>CONCURRENT Symposium: Exosome in Neurotherapeutics</p> <p>DESCRIPTION: Extracellular vesicles (EVs) have wide-ranging implications in numerous disease areas. Targeting these EVs as a therapeutic strategy is an ever increasing field. During this symposium speakers will look at EV biomarkers, EVs as therapeutics, and EVs for the clinical and preclinical diagnosis of Alzheimer's disease and other neurological and psychiatric diseases.</p> <p>CHAIR: Dimitrios Kapogiannis, MD, National Institute on Aging COCHAIR: Carolyn Tallon, PhD, ALTPEP</p> <p>FACULTY: Neuronal-enriched extracellular vesicles provide biomarkers predicting future cognitive impairment and AD Carlos Nogueras-Ortiz, PhD, National Institute on Aging</p> <p>Characterization of ATP1A3 as a neuron-specific EV protein and its potential applications for clinical diagnosis You Yang, PhD, Mayo Clinic College of Medicine and Science</p> <p>Inhibiting Extracellular Vesicle Biosynthesis as a Therapeutic Target for Alzheimer's Disease Meixiang Huang, PhD, Johns Hopkins Drug Discovery</p>
1:30pm - 3:00pm <i>Regency I</i>	<p>CONCURRENT Symposium: Digital Biomarkers in CNS clinical studies</p> <p>DESCRIPTION: The field of neuroscience is rapidly advancing with groundbreaking technologies and methodologies. In recent years, the emergence of digital biomarkers has revolutionized clinical studies in Central Nervous System disorders. This session will delve into the promising role of digital biomarkers in CNS clinical studies, exploring their potential to enhance diagnosis, monitoring, and treatment evaluation. During this session, esteemed experts in the field will present their latest research and share insights on the application of digital biomarkers across a range of CNS disorders. Attendees will gain a comprehensive understanding of the opportunities and challenges associated with leveraging digital biomarkers in clinical research.</p> <p>CHAIR: Bibi Bielokova, MD, NIAID COCHAIR: Sharon Tamir, MT-Pharma</p> <p>FACULTY: Developing Digital Technologies for CNS Clinical Studies: Regulatory Considerations Jay Gupta, PhD, CDRH, U.S. Food and Drug Administration (FDA)</p> <p>Wearable Sensors and Digital Health Technologies for Tracking Disease Progression in Neurological and Neuromuscular Disorders Dr. Ashkan Vaziri, PhD, Founder and CEO, BioSensics LLC</p> <p>Exploring Oculometrics as Digital biomarkers in CNS disorders Rivka Kreitman, PhD, NeuraLight</p>
3:00pm - 3:15pm <i>Regency Ballroom Foyer</i>	Networking Break

TUESDAY, MARCH 12, 2024

3:15pm - 5:00pm
Regency I

CONCURRENT Symposium: Opportunities to streamline development of gene therapies for rare diseases

DESCRIPTION: A significant percentage of rare diseases affecting 30 million people in the US alone are neurological, and 90% of rare childhood disorders have major neurological effects. Many rare diseases stem from single-gene variations, they may be addressable using rapidly maturing genetic technologies, such as antisense oligonucleotides, small interfering RNAs, gene therapy and gene editing. Recent advances in gene therapies have encouraged the scientific community to pursue these treatments as disease modifying treatments that potentially bring significant benefit to the patients. This symposium will address end-to-end regulatory path for individualized gene therapies

CHAIR: Gopa Raychaudhuri, PhD, Associate Director for Special Programs, FDA/CBER
COCHAIR: Amir Tamiz, PhD, NINDS

FACULTY:

Challenges in Bringing a Gene Therapy Product to the Global Marketplace: An Industry Perspective

Lawrence Stark, PhD, Novartis

Knowing your “why” can change the journey to gene therapy.

Gina Hann, Rare Village

Academic perspective on the development of gene therapy treatments for rare neurological disorders: How do we get these to patients?

Steven Gray, MD, UT Southwestern

Getting beyond “one disease at a time”: towards platform approaches to genetic therapies

Philip J. Brooks, PhD, NIH/NCATS

3:15pm - 5:00pm
Regency IV

CONCURRENT Symposium: Current Research and Advances in Multiple Sclerosis

DESCRIPTION: Microbial agents including viruses have long been incriminated as triggers in multiple sclerosis (MS). There is mounting evidence that EBV might play a role in MS pathogenesis. Stem cells transplantation therapy for MS is currently being evaluated in clinical trials including mesenchymal stem cell and autologous stem cells. The symposium will address advances in these three aspects of MS.

CHAIR: Suhayl Dhib-Jalbut, MD, Rutgers School of Medicine

COCHAIR: Steve Jacobson, PhD, NIH

FACULTY:

Title TBD

Steve Jacobson, PhD, NINDS

BTK Inhibitors as a Treatment for Chronic Inflammation in Multiple Sclerosis

María Inés Gaitán, MD, NIH

Glial Cells as Therapeutic Targets in Progressive Multiple Sclerosis

Marjan Gharagozloo, Ph.D., Johns Hopkins

5:00pm - 6:00pm
Regency Ballroom Foyer

Cocktail Reception

TUESDAY, MARCH 12, 2024

6:00pm - 8:00pm
Regency I

ASENT Careers Dinner

Each year ASENT invites a selection of speakers to informally share the trajectory of their career. We ask our speakers to come with no slides and a willingness to answer questions as there are many early career attendees in the audience.

Suhayl Dhib-Jalbut, MD, Rutgers School of Medicine
Gopa Raychaudhuri, PhD, Associate Director for Special Programs, FDA/CBER
Kevin Biglan, MD, MPH, Eli Lilly and Company

WEDNESDAY, MARCH 13, 2024

7:00am - 8:00am
Regency Ballroom Foyer

Registration and Breakfast

8:00am - 8:30am
Regency I

ASENT Business Meeting

8:30am - 10:00am
Regency I

Symposium: **Parkinson's disease biological staging and implications for therapeutic trials**

DESCRIPTION: Biological staging of Parkinson's disease is being proposed with use of imaging and cerebrospinal fluid biomarkers and prodromal clinical features. There is momentum in the field to define Parkinson's disease according to biological markers rather than traditional clinical features. This session seeks to explore the scientific, clinical, and regulatory issues surrounding pathways for therapeutics development using this framework.

CHAIR: Ludy Shih, MD, MMSc, FAAN, FANA, Boston University School of Medicine
COCHAIR: Kathleen Poston, M.D., M.S., Stanford University

FACULTY

Neuronal Synuclein Disease: Towards an Integrated Staging System to Accelerate Clinical Trials

Kathleen Poston, M.D., M.S., Stanford University

Perspectives, challenges and opportunities for clinical trials, research, and advocacy in Lewy body dementia

Jennifer Goldman, MD, MS, Lewy Body Dementia Association

Optimizing drug development in Lewy Body Diseases: Biological characterization of PD and lessons from Alzheimer's disease

Kevin Biglan, MD, MPH, Eli Lilly and Company

10:00am - 10:30am
Regency Ballroom Foyer

Networking Break

10:30am - 11:00am
Regency I

SPECIAL UPDATE: **Long-COVID and neurodegenerative diseases**

A major concern of the long-term consequences of COVID is the precipitation or acceleration of neurodegenerative diseases with cognitive dysfunction. The talk will review the current evidence, clinical and pathological manifestations, the pathophysiological mechanisms and potential modes of intervention. It will also discuss the need for future studies and clinical trials.

CHAIR: Debra J. Ehrlich, MD, MS, NINDS
COCHAIR: Sharon Tamir, Mitsubishi Tanabe Pharma America

FACULTY

Avindra Nath, MD, NINDS

11:00am - 12:00pm

MEETING OVERVIEW

WEDNESDAY, MARCH 13, 2024

12:00pm - 1:00pm <i>Regency IV</i>	SATELLITE SYMPOSIUM Clinilabs presents The Patient Perspective on Parkinson's Disease Clinical Research This satellite symposium will provide researchers with a direct patient perspective on participating in clinical trials. Patients will share their experiences and expectations to help guide the development of more patient-centric protocols in an effort to pave the way for improved enrollment and retention in Parkinson's clinical trials. This discussion will be led by: Angie Randazzo, PhD, Director, Patient Engagement & Advocacy, Clinilabs
12:00pm - 1:30pm <i>Regency I</i>	DEBATE: Amyloid Targeted Treatments for Alzheimer's Disease: The Arguments For and Against Collegial debate on the pros and cons of amyloid-targeting treatments for Alzheimer's disease with 4 speakers discussing the science supporting (or not) the validity of amyloid as a therapeutic target and the data from the completed RCTs of newly approved amyloid antibodies (Lecanemab and Aducanumab) with a focus on the gaps in clinical data and the assessment of risks vs. benefits. CHAIR: Madhav Thambisetty, MD, PhD, NIH COCHAIR: Amir Tamiz, PhD, NIH A rapid fire among 4 clinician scientists, up to 10 minutes each: FOR: John Dickson, MD, PhD, Harvard AGAINST: Alberto Espay, MD, University of Cincinnati FOR: David C. Weisman, MD, Abington Neurological Associates AGAINST: Madhav Thambisetty, MD, PhD, NIH To be followed by rebuttals and a robust interactive discussion.
1:30pm - 3:00pm <i>Regency I</i>	Pipeline Presentations Submitted abstracts that most closely conform to the ASENT Abstract criteria, showcase new and emerging data with scientific or clinical impact and align with ASENT goals have been selected for a 10-minute oral presentation (including 5 minute Q&A) at one of our Pipeline Presentation sessions. Improvement in a plasma biomarker of neuroinflammation, Chitinase-3-like-protein (CHI3L1/YLK-40), in the CENTAUR trial Presenter: Heather Vita, PhD, AMYLYX Pharmaceuticals Bilateral intrastratial delivery of Life Edit CRISPR system (LEG-B) using AAV5 results in broad CNS biodistribution and expression of LEG-B in NHP brain regions critical for Huntington's disease (HTT) pathology, and allele selective reduction of mutant HTT protein in a clinically relevant murine model Presenter: Hanqian Mao, PhD, Life Edit Therapeutics Effects of GLP-1 receptor agonist on chronic axonal damage, neurodegeneration, and cognitive function in relapsing-remitting MS (MSGP): exploratory, open label, randomized controlled trial. Presenter: Richard Imrich, PhD, DSc, Biomedical Research Center, Slovak Academy of Sciences BAER-101, a selective potentiator of $\alpha 2$ - and $\alpha 3$ -containing GABAA receptors, fully suppresses spontaneous cortical spike-wave discharges in Genetic Absence Epilepsy Presenter: Amy Chappell, M.D., Avenue Therapeutics

MEETING OVERVIEW

WEDNESDAY, MARCH 13, 2024

3:00pm - 3:30pm <i>Regency Ballroom Foyer</i>	Networking Break
3:30pm - 5:00pm <i>Regency I</i>	<p>Platform Presentations</p> <p>Submitted abstracts that most closely conform to the ASENT Abstract criteria, showcase novel design or concepts and align with ASENT goals may also be selected for a 10-minute oral presentation (including 2 minute Q&A) at one of our Platform Presentation sessions.</p> <p>Positive allosteric modulation of GABAA $\alpha 5$ receptors improves memory performance of rats with conditions of hippocampal hyperactivity Presenter: Ming-Teng Koh, Ph.D., Johns Hopkins University</p> <p>Baseline network predictor of the placebo response: Can susceptible individuals be identified before randomization? Presenter: János A. Barbero, The Feinstein Institutes for Medical Research</p> <p>Bridging translational research to clinical research provides therapeutic opportunities to patients with a primary mitochondrial disease Presenter: Anne Chiamello, Ph.D., George Washington University School of Medicine and Health Sciences</p> <p>ALT100, a designed peptide targeting α-sheet Amyloid-β oligomers, improves behavior in an ICV oligomeric Aβ mouse model Presenter: Carolyn Tallon, Ph.D., AltPep Corp.</p> <p>Development of novel small molecules targeting neurotrophic HGF signaling for the treatment of Alzheimer's, Parkinson's, and ALS Presenter: Robert Taylor, Ph.D., Athira Pharma, Inc.</p>
5:00pm - 6:00pm <i>Regency Ballroom Foyer</i>	Cocktail and Poster Reception

MEETING OVERVIEW

WEDNESDAY, MARCH 13, 2024

6:00pm - 8:00pm

Regency I

Neurotherapeutics Dinner

Guest Speaker: Irfan Qureshi, M.D., CMO, Biohaven

This dinner symposium is a combined effort of the Training in Neurotherapeutics Discovery and Development Course and the ASENT2024 Annual Meeting. All participants are welcome to attend.

CHAIR: Michael Rogawski, MD, PhD, University of California, Davis

CO-CHAIR: Andrew J. Cole, MD, F.R.C.P.(C.) Harvard Medical School/MGH

THURSDAY, MARCH 14, 2024

7:00am - 8:20am

Regency Ballroom Foyer

Registration and Breakfast

8:20am - 8:30am

Regency I

Presidential Closing Day Remarks

8:30am - 10:00am

Regency I

Presidential Symposium: Pre-clinical to First in Human In Drug Development

DESCRIPTION: This fireside chat will feature speakers from both a small independent academic startup as well as a speaker from a large pharmaceutical company to compare and contrast the similarities and differences in the preclinical process and infrastructure when developing a neurotherapeutic. This discussion will be moderated by ASENT President Andrew Cole, MD.

CHAIR: Nathan Fountain, MD, University of VA School of Medicine

CO-CHAIR: Andrew J. Cole, MD, F.R.C.P.(C.) Harvard Medical School/MGH

FACULTY:

Nathan Fountain, MD, University of Virginia

Jim Doherty, PhD, Acumen Pharmaceuticals, Inc.

10:00am - 10:30am

Regency Ballroom Foyer

Networking Break

10:30am -12:00 pm

Regency I

CONCURRENT Symposium: Novel Delivery Systems in CNS

DESCRIPTION: Oligonucleotides, antibodies, and gene therapies are increasingly poised to provide significant benefit for central nervous system (CNS) disorders. These modalities offer great promise to change the course of a disease. However, delivery of these therapeutics remains a significant challenge as they do not readily penetrate the blood brain barrier. Informed by fundamental multidisciplinary research in blood-brain barrier biology, protein engineering is identifying solutions that enable the delivery of these therapeutic modalities into the CNS. This symposium will highlight several innovative approaches to improve biotherapeutics delivery to the brain.

CHAIR: Ben Deverman, PhD, MIT and Harvard

CO-CHAIR: Amir Tamiz, PhD, NINDS

FACULTY:

Transport Vehicle: Utilizing the Brain Vasculature for Delivering CNS Therapeutics

Joy Zuchero, PhD, Denali Therapeutics

AAVs engineered to bind Transferrin Receptor enable CNS-wide gene delivery

Ben Deverman, PhD, Broad Institute of MIT and Harvard

Discovery and mechanism of action of cross-species BBB-penetrant capsids

Mathieu Nonnenmacher, PhD, Voyager Therapeutics

MEETING OVERVIEW

THURSDAY, MARCH 14, 2024

10:30am -12:00 pm
Regency IV

CONCURRENT Symposium: **Animal Models in Neurotherapeutics: the good, the bad and the ugly.**

DESCRIPTION: A crucial component to drug discovery research relies on utilizing animal models to demonstrate efficacy in disease specific models. However, the choice of model to use has always been a difficult question to navigate given the wide variety of available models for any given neurological disease. This symposium aims to discuss the common pitfalls to avoid when utilizing animal models and provide discussion on what to consider when designing animal model experiments for drug development in the neurological space.

CHAIR: Carolyn Tallon, PhD, AltPep

FACULTY:

Perspectives and Paths Forward for overcoming the Preclinical to Clinical Translation conundrum in Alzheimer's disease Therapy Development

Stacey J. Sukoff Rizzo, PhD, University of Pittsburgh

Animal Models for Drug Development in Epilepsy: Challenges and Opportunities

Cameron Metcalf, PhD, Anticonvulsant Drug Development Program, University of Utah

Why have all clinical trials in PD failed: are we using the wrong animal model or study design, or both?

Charles K. Meshul, PhD, Oregon Health and Science University

Meeting Concludes

2024 POSTERS IN ORDER BY POSTER # 1-7 PLATFORM PRESENTATIONS

#1

BAER-101, a selective potentiator of $\alpha 2$ - and $\alpha 3$ -containing GABAA receptors, fully suppresses spontaneous cortical spike-wave discharges in Genetic Absence Epilepsy

Presenter: Amy Chappell, M.D., Avenue Therapeutics

Abstract Authors: Amy Chappell, Alexandra MacLean, Corinne Roucard, Alexis Evrard, Hugo Monchal

Platform Presentation

#2

Positive allosteric modulation of GABAA $\alpha 5$ receptors improves memory performance of rats with conditions of hippocampal hyperactivity

Presenter: Ming-Teng Koh, Ph.D., Johns Hopkins University

Abstract Authors: Ming Teng Koh, Sharon Rosenzweig-Lipson, Michela Gallagher

Platform Presentation

#3

Baseline network predictor of the placebo response: Can susceptible individuals be identified before randomization?

Presenter: János A. Barbero, The Feinstein Institutes for Medical Research

Abstract Authors: János Barbero, An Vo, Nha Nguyen, Yilong Ma, Shichun Peng, Chris Tang, Charalampos Tzoulis, David Eidelberg

Platform Presentation

#4

Bridging translational research to clinical research provides therapeutic opportunities to patients with a primary mitochondrial disease

Presenter: Anne Chiaramello, Ph.D., George Washington University School of Medicine and Health Sciences

Abstract Authors: Martine Uittenbogaard, Andrea Gropman, Anne Chiaramello

Platform Presentation

#5

ALT100, a designed peptide targeting α -sheet Amyloid- β oligomers, improves behavior in an ICV oligomeric A β mouse model

Presenter: Carolyn Tallon, Ph.D., AltPep Corp.

Abstract Authors: Carolyn Tallon, Chandresh Gajera, Chris Tran, Jeff Posakony, Charles Watt, Gill Block, Valerie Daggett

Platform Presentation

#6

Antisense oligonucleotides directed against a human endogenous retrovirus for treatment of Amyotrophic Lateral Sclerosis

Presenter: Kevon Sampson, National Institute of Neurological Disorders and Stroke, National Institutes of Health

Abstract Authors: Kevon Sampson, Wenxue Li, Myoung Hwa Lee, Lisa Henderson, Avindra Nath

Poster Presentation

#7

Development of novel small molecules targeting neurotrophic HGF signaling for the treatment of Alzheimer's, Parkinson's, and ALS

Presenter: Robert Taylor, Ph.D., Athira Pharma, Inc.

Abstract Authors: Robert Taylor, Andree-Anne Berthiaume, Kayla Kleist, Sherif Reda, Sharay Setti, Jewel Johnston, Wei Wu, Kevin Church

Platform Presentation

MEETING OVERVIEW

2024 POSTERS IN ORDER BY POSTER # 8-13 PIPELINE PRESENTATIONS

#8

Safety and Tolerability of BHV-7000, a Novel Kv7 Potassium Channel Activator: Results from Phase 1 Single and Multiple Ascending Dose Studies

Presenter: Irfan Qureshi, M.D., Biohaven Pharmaceuticals

Abstract Authors: Bharat Awsare, Jason Lerner, Eric Ashbrenner, Heather Sevinsky, Michael Bozik, Steven Dworetzky, Lia Donahue, Randall Killingsworth, Bruno Francoeur, Irfan Qureshi

Poster Presentation

#9

BHV-7000, A Novel, Selective Kv7.2/7.3 Potassium Channel Activator, Demonstrates Dose-Dependent Pharmacodynamic Effects on EEG Parameters in Healthy Adults

Presenter: Irfan Qureshi, M.D., Biohaven Pharmaceuticals

Abstract Authors: Jason Lerner, Bharat Awsare, Heather Sevinsky, Eric Ashbrenner, Randall Killingsworth, Racheal Kendrick, Emiel Vereycken, Nigel Colenbier, Caroline Neuray, Pieter van Mierlo, Jeremy Slater, David Wyatt, Irfan Qureshi, Steven Dworetzky, Michael Bozik

Poster Presentation

#10

Improvement in a plasma biomarker of neuroinflammation, Chitinase-3-like-protein (CHI3L1/YLK-40), in the CENTAUR trial

Presenter: Heather Vita, Ph.D., AMYLYX Pharmaceuticals

Abstract Authors: Robert Bowser, Jiyan An, Lahar Mehta, Junliang Chen, Jamie Timmons, Merit Cudkowicz, Sabrina Paganoni, Heather Vita

Pipeline Presentation

#11

Bilateral intrastratial delivery of Life Edit CRISPR system (LEG-B) using AAV5 results in broad CNS biodistribution and expression of LEG-B in NHP brain regions critical for Huntington's disease (HTT) pathology, and allele selective reduction of mutant HTT protein in a clinically relevant murine model

Presenter: Hanqian Mao, Ph.D., Life Edit Therapeutics

Abstract Authors: Hanqian Mao, Logan Brown, Nancy Cheng, Alexandra Crawley, Ritika Jaini, Jamie Moy, Ariel Vitenzon, Kathryn Woodburn

Pipeline Presentation

#12

Effects of GLP-1 receptor agonist on chronic axonal damage, neurodegeneration, and cognitive function in relapsing-remitting MS (MSGLP): exploratory, open label, randomized controlled trial.

Presenter: Richard Imrich, Ph.D., DSc., Biomedical Research Center, Slovak Academy of Sciences

Abstract Authors: Richard Imrich, Adela Penesová, Miroslav Vlček, Žofia Rádiková, Andrea Havranová, Pavol Šiarnik, Miroslava Harďonová, Branislav Kollár, Peter Turčáni

Pipeline Presentation

#13

From standardization to specificity: a retrospective analysis of adverse event classification in neurological clinical research

Presenter: Katherine Landry, National Institutes of Health

Abstract Authors: Katherine Landry, Matthew Gooden, Sandra Martin, Henry Roberts, Lauren Reoma

Poster Presentation

MEETING OVERVIEW

2024 POSTERS IN ORDER BY POSTER # 14-20

#14

Active Neurogenic Modulation of Cerebral Blood Flow to Alter Flow Dynamics in Patients with Neurodegenerative Diseases and in Aging Adults

Presenter: Robert D. Black, Ph.D., Scion Neurostim

Abstract Authors: Robert D. Black, Ph.D.

Poster Presentation

#15

Investigator-initiated Clinical Trials for Neurological Diseases in Japan: Pipeline and Support Structures

Presenter: Ken Sakushima, M.D., M.P.H., Ph.D., Hokkaido University Hospital

Abstract Authors: Ken Sakushima, Harumasa Nakamura, Kazuo Fujihara

Poster Presentation

#16

AlzPED: An Open Science Tool Raising the Standards for Preclinical Testing of Candidate Therapeutics in Alzheimer's Disease Animal Models.

Presenter: Jaya Viswanathan, Ph.D., National Institute on Aging, National Institutes of Health

Abstract Authors: Jaya Viswanathan, Maria Fe Lanfranco Gallofre, Zane Martin, Suzana Petanceska, Shreaya Chakroborty, Lorenzo Refolo

Poster Presentation

#17

Development of a Composite Biomarker for Amyotrophic Lateral Sclerosis: Experimental Approach and Progress to Date

Presenter: Heather Vita, Ph.D., AMYLYX Pharmaceuticals

Abstract Authors: Jamie Timmons, Evan Mizerak, Joshua Cohen, Justin Klee, Sasha Bakhru, James Berry, Robert Bowser, Heather Vita

Poster Presentation

#18

AGB101 for the treatment of neuropsychiatric symptoms in Parkinson's disease

Presenter: Arnold Bakker, Ph.D., Johns Hopkins University

Abstract Authors: Arnold Bakker, Gregory Pontone, Richard Mohs, Michela Gallagher

Poster Presentation

#19

Decoding therapeutic signatures: The contribution of pharmaco-EEG in profiling compounds with varied mechanisms of action.

Presenter: Julien Volle, Ph.D., SynapCell

Abstract Authors: Julien Volle, Manon Villalba, Clélia Allieux, Baptiste Caraballo, Carine Dumont, Eloïse Gronlier, Davy Darankoum, Corinne Roucard, Yann Roche, Chloé Habermacher

Poster Presentation

#20

Translational Evaluation of Acute 4-Aminopyridine (4-AP) Treatment to Reduce Axon Damage after Experimental Traumatic Brain Injury

Presenter: Kryslaine L. Radomski, Ph.D., Uniformed Services University of the Health Sciences

Abstract Authors: Kryslaine Radomski, Xiaomei Zi, Regina Armstrong

Poster Presentation

MEETING OVERVIEW

2024 POSTERS IN ORDER BY POSTER # 21-27

#21

GlyB4: A novel biologic to stop neuroinflammation and neurodegeneration in ALS and Alzheimer's disease

Presenter: Jeffrey A. Loeb, M.D., Ph.D., University of Illinois at Chicago

Abstract Authors: Jeffrey Loeb, Michael Flavin, Fei Song

Poster Presentation

#22

Human endogenous retrovirus-K (HERV-K) env knockdown by AAV9-mediated shRNA attenuates amyotrophic lateral sclerosis (ALS)-like symptoms in transgenic mice

Presenter: Myoung-Hwa Lee, Ph.D., National Institute of Neurological Disorders and Stroke, National Institutes of Health

Abstract Authors: Myoung-Hwa Lee, Wenxue Li, Kevon Sampson, Melina Jones, Joseph Steiner, Avindra Nath

Poster Presentation

#23

Beyond the diagnosis: Advancing quality of life for those with progressive diseases through innovative housing and technology solutions.

Presenter: Theresa Whitlock-Wild, Matt's Place Foundation

Abstract Author: Theresa Whitlock-Wild

Poster Presentation

#24

Repurposing on old drug for a new disease-modifying therapy for epileptic disorders with brain calcifications

Presenter: TJ Stalvey, M.P.H., M.B.A., ZY Therapeutics

Abstract Authors: TJ Stalvey, Jeffrey Loeb

Poster Presentation

#25

The NIH HEAL Initiative/National Institute of Neurological Disorders and Stroke's Early Phase Pain Investigation Clinical Network (EPPIC-Net): Year 4 Update.

Presenter: Rebecca Hommer, M.D., National Institutes of Health

Abstract Authors: Rebecca Hommer, Kevin Jones, Marlene Peters-Lawrence, Clinton Wright

Poster Presentation

#26

Novel mitochondrial protective agent for Parkinson's Disease targeting the PINK1/LRRK2 axis

Presenter: Sherine S. Chan, Ph.D., Neuroene Therapeutics

Abstract Authors: Sherine Chan, Emmaline Bendell, Jesse McClure, James Chou

Poster Presentation

#27

The NIH HEAL Initiative Preclinical Screening Platform for Pain (PSPP)

Presenter: Sarah A. Woller, Ph.D., National Institute of Neurological Disorders and Stroke, National Institutes of Health

Abstract Authors: Sarah Woller, Shalini Sharma, Christopher Conrad, Vicki Brings, Smriti Iyengar

Poster Presentation

MEETING OVERVIEW

2024 POSTERS IN ORDER BY POSTER # 28-31

#28

Neuropathology Evaluation of Olney Lesions in Regulatory Drug Development Toxicology Studies

Presenter: Deepa B. Rao, BVSc, M.S., Ph.D., Greenfield Pathology Services, Inc.

Abstract Authors: Deepa Rao, David Hawver, David Hall, William Jordan

Poster Presentation

#29

NeuroNEXT: A Summary of the First 12 Years & A Look Towards the Future

Presenter: Christopher S. Coffey, Ph.D., University of Iowa

Abstract Authors: Christopher Coffey, Megan McCabe, Aleks Videnovic, Dixie Ecklund, Marianne Chase, Joan Ohayon, Sophie Cho, Amy Tsou, Brenda Thornell, David Klements, Michele Costigan, Maria Houghton, Michael Moshinsky, Emily Roberts, Merit Cudkowicz

Poster Presentation

#30

A phase 2 trial of T-DXd for tumor-agnostic HER2-positive brain and leptomeningeal metastases

Presenter: Alice R. Tang, MBBS, Dana–Farber Cancer Institute

Abstract Author: Alice Tang

Poster Presentation

#31

The NIH HEAL Pain Therapeutics Development Program RFA-NS-24-019

Presenter: Matthew Rice, Ph.D., National Institute of Neurological Disorders and Stroke, National Institutes of Health

Abstract Authors: Matthew Rice, Carolyn Bondar, Mary Ann Pelleymounter

Poster Presentation

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Abstract Deadline

January 22, 2024

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Early Bird Deadline

December 31, 2023

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Hotel Reservation Deadline

February 10, 2024

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