



Neuro Gene Therapy Symposium

Thursday and Friday
October 12 – 13, 2023
Gaulton Auditorium, BRB
University of Pennsylvania

Day 1: Thursday, October 12, 2023

08:00 am – 09:00 am Check-in & Breakfast

09:00 am – 09:15 am Welcome & Introductory Remarks
Frances Jensen, MD & Raquel Gur, MD, PhD; Co-Directors, PTNC

Session I: Introduction to Gene Therapy & Current Scope – Chair: Frances Jensen, MD

09:15 am – 09:45 am **Kenneth (Kurt) Fischbeck, MD**, NIH Distinguished Investigator, National Institute of Neurological Disorders and Stroke (NINDS)
Diagnosis and Treatment of Hereditary Neurological and Neuromuscular Diseases

09:45 am – 10:15 am **Daniel J. Rader, MD**, Seymour Gray Professor of Molecular Medicine; Chair of the Department of Genetics Chief of the Division of Translational Medicine and Human Genetics in the Department of Medicine, University of Pennsylvania
Translational Resources for Genetics at Penn

10:15 am – 11:00 am **Joint Talk:**

Lauren Elman, MD, Professor of Neurology, Director of the Muscular Dystrophy Association Clinic; Medical Center Director of the Penn Comprehensive Amyotrophic Lateral Sclerosis Center, University of Pennsylvania
Two Clinics Separated By 20 Years & a Cautionary Tale

Ingo Helbig, MD, Assistant Professor, Division of Neurology, Children's Hospital of Philadelphia and the Clinical Director, Center for Epilepsy and Neurodevelopmental Disorders (ENDD) at University of Pennsylvania and Children's Hospital of Philadelphia
Clinical Trial Readiness and Natural History in Pediatric Epilepsies and Neurodevelopmental Disorders

11:00 am - 11:15 am: Break

Session II: Successes & Current Landscape - Chair: Steven Scherer, MD, PhD

- 11:15 am – 11:45 am** **Jean Bennett, MD, PhD**, F.M. Kirby Professor of Ophthalmology, Department of Cell and Developmental Biology and Vice Chair for Basic Research, Department of Ophthalmology; Director of DNA, Tissue and Cell Line Bank for Retinal Degeneration & Molecular Diagnostic Studies Unit for Patients with Retinal Degeneration, F. M. Kirby Center for Molecular Ophthalmology, University of Pennsylvania
The Luxturna Experience: Insights Into Development of Gene-Based Treatments for Other Blinding Disorders (pre-recorded talk)
- 11:45 am – 12:15 pm** **Toby Ferguson, MD, PhD**, Head Neuromuscular and Movement Disorders Development Unit, Biogen
Use of Neurofilament in Development of Tofersen for SOD1 ALS
- 12:15 pm – 12:45 pm** **Steven Scherer, MD, PhD**, Ruth Wagner Van Meter and J. Ray Van Meter Professor of Neurology, Director of Penn Neurogenetics Therapy Center, University of Pennsylvania
Successful Treatments for Transthyretin Amyloidosis (ATTR)
- 12:45 pm – 01:15 pm** **Kevin Flanigan, MD**, Robert F. and Edgar T. Wolfe Foundation Endowed Chair in Neuromuscular Research; Director, Center for Gene Therapy at the Abigail Wexner Research Institute, Nationwide Children’s Hospital
Next Directions in Dystrophinopathy Gene Therapy

01:15 pm – 2:15 pm: Lunch/Break

Session III: Different Methodologies/Mechanisms – Chair: Frances Jensen, MD

- 02:15 pm – 02:45 pm** **Beverly L. Davidson, PhD**, Katherine A. High Chair in Cell and Gene Therapy, Director, Raymond G. Perelman Center for Cellular and Molecular Therapeutics, Chief Scientific Strategy Officer, Children’s Hospital of Philadelphia and Professor of Pathology and Laboratory Medicine, University of Pennsylvania
Advancing Brain Gene Therapies
- 02:45 pm – 03:15 pm** **Drew Weissman, MD, PhD, Nobel Laureate**
Roberts Family Professor in Vaccine Research; Director, Penn Institute for RNA Innovation; Director, Vaccine Research in the Infectious Disease Division; University of Pennsylvania
2023 Nobel Prize in Physiology or Medicine
Nucleoside-Modified mRNA-LNP Therapeutics
- 03:15 pm – 03:45 pm** **Ricardo Dolmetsch, PhD**, President and Chief Scientific Officer, uniQure
Developing a Gene Therapy to Treat Huntington’s Disease

03:45 pm – 4:00 pm: Break

Session IV: Frontiers & Vision Forward – Chair: Raquel Gur, MD, PhD

04:00 pm – 04:30 pm **Benjamin L. Prosser, PhD**, Associate Professor, Physiology; Director, Center for Epilepsy and Neurodevelopmental Disorders (ENDD); Associate Director, Pennsylvania Muscle Institute; Lead Coordinator, Leducq Transatlantic Network of Excellence (Leducq Cytoskeletal Network), University of Pennsylvania
Developing and Accelerating Therapeutics for Rare Neurodevelopmental Disorders (pre-recorded talk)

04:30 pm – 05:00 pm **Steven E. Hyman, MD**, Core Institute Member, Director of the Stanley Center for Psychiatric Research, Broad Institute at MIT and Harvard
Schizophrenia: The Challenging Path from Genetics to Therapeutics

Day 2: Friday, October 13, 2023

08:00 am – 08:30 am **Check-in & Breakfast**

Session V: Academic Gene Therapy Centers - Chair: Colin Quinn, MD

08:30 am – 09:00 am **Sara-Claude Michon, PhD**, Director of Operations, Penn Neurogenetics Therapy Center, University of Pennsylvania
Amy T. Waldman, MD, MSCE, Associate Director, Clinical In Vivo Gene Therapy, Children’s Hospital of Philadelphia
Joint talk: Setting Up an Academic Gene Therapy Center: Penn Neurogenetics Therapy Center and CHOP Clinical In Vivo Gene Therapy Center

09:00 am – 09:30 am **James Wilson, MD, PhD**, Director, Gene Therapy Program; Rose H. Weiss Professor and Director, Orphan Disease Center; Professor of Medicine and Pediatrics, University of Pennsylvania
A Penn-Based Platform for Global Delivery of AAV Vectors to CNS

09:30 am – 10:00 am **Pedro Gonzalez-Alegre, MD, PhD**, Head of Gene Therapy Research, Spark Therapeutics, Inc
From Academia to Industry, and Back

10:00 am – 10:15 am: Break

Session VI: Ethics & Regulatory Issues – Chair: Lauren Elman, MD

10:15 am – 10:45 am **Holly Tabor, PhD**, Associate Professor of Medicine at Stanford University; Associate Director for Clinical Ethics and Education for the Stanford Center for Biomedical Ethics (SCBE), Co-Chair of the Ethics Committees at Stanford Hospital and Lucile Packard Children’s Hospital
Ethical Issues of Gene Therapy

10:45 am – 11:15 am **Peter Marks, MD, PhD**, Director of the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration (FDA)
Facilitating the Development and Availability of Gene Therapy for Small Populations

11:15 am – 11:45 am **Allyson Berent, DVM, DACVIM**, Chief Science Officer, Foundation for Angelman Syndrome Therapeutics (FAST); Co-Director, Angelman Syndrome Biomarker and Outcome Measure Consortium; Chief Development Officer, Mahzi Therapeutics
A Journey Through Drug Development: How parents and patients are taking the lead: The Angelman Story of making the impossible possible

11:45 am – 12:00 pm: Break

Session VII: Future Directions & Emerging Targets – Chair: Stewart Anderson, MD

12:00 pm – 12:30 pm **David Irwin, MD**, Assistant Professor, Neurology; Co-Director, Penn Frontotemporal Degeneration Center, University of Pennsylvania
Hope Renewed: Gene Therapy Research for FTD
Caregiver speaker – recorded talk with live Q&A

12:30 pm – 01:00 pm **Erik Roberson, MD, PhD**, Professor, Rebecca Gale Endowed Professor, Vice Chair for Basic & Translational Research, Department of Neurology; Director, Alzheimer's Disease Center; Director, Center for Neurodegeneration and Experimental Therapeutics, University of Alabama at Birmingham
Optimizing Progranulin Gene Therapy for Frontotemporal Dementia Using Preclinical Models

01:00 pm – 01:30 pm **Michael Kaplitt, MD, PhD**, Professor of Neurological Surgery; Residency Director and Vice Chairman for Research, Weill Cornell Medical College
Opportunities and Challenges for Gene Therapies in Neurodegenerative Diseases

Concluding Remarks **Raquel Gur, MD, PhD & Frances Jensen, MD; Co-Directors, PTNC**

take away lunch available

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