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  Kurt Fischbeck, MD, Chief of the Neurogenetics Branch, NINDS
Topic: Diagnosis and treatment of hereditary neurological and neuromuscular diseases

09:45 am – 10:15 am  Daniel Rader, MD, Chair, Department of Genetics, University of Pennsylvania
Topic: Neurogenetics in context of overall field of genetics

10:15 am – 11:00 am  Ingo Helbig, MD, Assistant Professor, Division of Neurology and the Director of Genomic Science at Children's Hospital of Philadelphia
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
Topic: Joint talk on the role of neurogenetic diagnostics and natural history studies – pediatrics to adult

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
Topic: Luxturna (pre-recorded talk, cannot attend)

11:45 am – 12:15 pm  
**Toby Ferguson, MD, PhD,** Vice President, Head Neuromuscular Development Unit, Biogen  
**Topic:** ALS & SMA - Spinraza or zolgensma

12:15 pm – 12:45 pm  
**Steven Scherer, MD, PhD,** Ruth Wagner Van Meter and J. Ray Van Meter Professor of Neurology, Interim Director of Penn Neurogenetics Therapy Center, University of Pennsylvania  
**Topic:** TTR Amyloidosis/neuropathies

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, Nationwide Children’s Hospital  
**Topic:** DMD

01:15 pm – 2:00 pm: Lunch/Break

**Session III: Different Methodologies/Mechanisms – Chair: TBD**

02:00 pm – 02:30 pm  
**Beverly Davidson, PhD,** Director, Raymond G. Perelman Center for Cellular and Molecular Therapeutics, Chief Scientific Strategy Officer, and Arthur V. Meigs Chair in Pediatrics at Children’s Hospital of Philadelphia and Professor of Pathology and Laboratory Medicine, University of Pennsylvania  
**Topic: Intro including other mechanisms (non-AAV, CRISPR)**

02:30 pm – 03:00 pm  
**Drew Weissman, MD, PhD,** Roberts Family Professor in Vaccine Research, Director of Vaccine Research, Infectious Diseases Division and Director, Institute for RNA Innovation, University of Pennsylvania  
**Topic:** mRNA

03:00 pm – 03:30 pm  
**Ricardo Dolmetsch, PhD,** President and Chief Scientific Officer, uniQure  
**Topic:** Implementation of emerging technologies by industry

03:30 pm – 3:45 pm: Break

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

03:45 pm – 04:15 pm  
**Benjamin L. Prosser, PhD,** Associate Professor, Physiology; Director, Center for Epilepsy and Neurodevelopmental Disorders (ENDD); Associate Director, Pennsylvania Muscle Institute; University of Pennsylvania  
**Topic:** Neurodevelopment, Rare Diseases and N of 1 trials; What does it take, where, accelerated pathway for translation from bench to bedside (pre-recorded talk, cannot attend)

04:15 pm – 04:45 pm  
**Steven Hyman, MD,** Core Institute Member, Director of the Stanley Center for Psychiatric Research, Broad Institute at MIT and Harvard  
**Topic:** Genetic Therapies in psychiatric disorders; Visioning Forward
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
*Topic: Setting up an academic gene therapy center; Penn’s Neurogenetics 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
*Topic: Challenges to Implementation*

09:30 am – 10:00 am  Pedro Gonzalez-Alegre, MD, PhD, Head of Gene Therapy Research, Spark Therapeutics, Inc
*Topic: Pharma perspective as partner*

10:00 am – 10:15 am: Break

Session VI: Ethics & Regulatory Issues – Chair: TBD

10:15 am – 10:45 am  Holly Tabor, PhD, Stanford -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
*Topic: 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)
*Topic: Regulatory Issues/FDA Perspective*

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
*Topic: Patient advocacy group/foundation*

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
**Topic: Intro to FTD gene therapy trials & caregiver speakers (1-2)**

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  
*Topic: Preclinical Models*

01:00 pm – 01:30 pm  **Michael Kaplitt, MD, PhD,** Professor of Neurological Surgery, Neurology, Department and Neurological Surgery (WCMC); Professor of Neuroscience, Department Brain and Mind Research Institute (WCMC); Professor of Neurological Surgery in Otolaryngology, Weill Cornell Medicine  
*Topic: Neurodegeneration/Parkinson's*

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

*take away lunch available*