FTD Treatment Study Group 2021
Innovations and Progress in Clinical Trial Design for FTD
December 8 & 9, 2021
Participants welcomed in Silver Spring, MD, USA or online

Day 1
7:30-8:40am ET: In-person breakfast

9:00am ET: Welcome
Susan Dickinson MSGC, AFTD
Penny Dacks PhD, AFTD
Billy Dunn MD, FDA Office of Neuroscience

9:10-12:45pm ET: SESSION 1, The Therapeutic Landscape for FTD
Chair: Howard Feldman MD FRCP(C), UCSD

• 9:15 - 5m: Welcome – Opening remarks

• 9:20 - 20m: Keynote: Lessons learned in targeting familial disease
  C. Frank Bennett PhD, Ionis Pharmaceuticals Inc.

• 9:40 - 60m: Lightning Round: industry trials for familial FTD
  • Sam Jackson MD, MBA, Alector
  • Richard Tsai MD, Denali Therapeutics
  • Tiffini Voss MD, PassageBio
  • Olga Uspenskaya MD, PhD, Prevail Therapeutics
  • Andrew Satlin MD, Transposon Therapeutics
  • Kenechi Ejebe MD, WaveLife Sciences
  10 min Q&A

• 10:40 - 15m: Break

• 10:55 - 15m: Landscape of the FTD Biomarkers
  Nicholas Ashton PhD, University of Gothenburg

• 11:10 - 25m: Neurofilament and composite biomarkers
  10m - Neurofilament light: perspectives across neurodegenerative disease
  Terina Martinez PhD, Critical Path Institute
  10m - Nfl and combination biomarkers for FTD
Julio Rojas-Martinez MD, PhD, UCSF

5m Q&A:

• 11:35 - 25m: Moderated discussion: Biomarkers for current and future FTD clinical trials
  Moderator (1 to facilitate virtual comments, 1 live)
  Primary discussion moderator – Nicholas Ashton PhD, University of Gothenburg
  Secondary discussion moderator – Howard Feldman MD, FRCP(C), UCSD
  Panelists –
  • Ramakrishna Boyanapalli PhD, WaveLife Sciences
  • Andrew Satlin MD, Transposon Therapeutics
  • Olga Uspenskaya MD, PhD, Prevail Therapeutics
  • Mark Forman MD PhD, Passage Bio
  • Kimberly Scearce-Levie PhD, Denali Therapeutics
  • Sam Jackson MD, MBA, Alector

• 12:00 - 45m: Moderated discussion – Perspectives on benefit and risk for FTD treatments
  Moderators (1 to facilitate virtual comments, 1 live)
  Primary discussion moderator – session chair, Howard Feldman MD, FRCP(C), UCSD
  Secondary discussion moderator – Penny Dacks PhD, AFTD
  Panelists –
  Community perspective: EL-PFDD & FTD Insights Survey – Shana Dodge PhD, AFTD
  Dementia and Family Advocate - Wanda Smith
  Bioethicist – Jason Karlawish MD, University of Pennsylvania
  Levels of safety monitoring perspective – David Knopman MD, Mayo Clinic
  Regulatory expert – Lucas Kempf MD, Parexel

• 12:45-1:45: Lunch (1HR)

1:45-5pm ET: SESSION 2 – Trial Design 2.0
Chair: Michael Gold MS, MD Abbvie

• 1:45 - 5m: Opening remarks
  Michael Gold MS, MD, Abbvie

• 1:50 - 10m: Treatment development for rare disease
  Amir Tamiz PhD, NINDS Division of Translational Research

• 2:00 - 15m: The rights of precision drug development
  Jeffrey Cummings MD, ScD, University of Nevada Las Vegas

• 2:15 - 15m: Update from the FTD Prevention Initiative (10m/5m Q&A)
Jon Rohrer PhD, MRCP, University College London

- **2:30 - 15m**: Disease modeling for historical controls (10m/5m Q&A)
  Adam Staffaroni PhD, UCSF

- **2:45 - 15m**: Digital twins and novel statistical approaches to trial design – (10m/5m Q&A)
  Charles Fisher PhD, UnlearnAI

- **3:00 - 15m**: Finding patients earlier: the potential of biomarker combinations (10m/5m Q&A)
  Holly Soares PhD, Pfizer

- **3:15 - 15m**: Break

- **3:30 - 30m**: Platform trials – learning from related fields
  Healey ALS Platform Trial – Sabrina Paganoni MD, PhD, MGH
  DIAN-TU Platform – Eric McDade DO, Washington University
  Q&A

- **4:00 - 60m**: Panel discussion: Trial design for success in rare disease
  Moderator (1 to facilitate virtual comments, 1 live)
  Primary moderator – Michael Gold MS, MD, Abbvie (session chair)
  Secondary moderator – Debra Niehoff, PhD, AFTD
  Panelists – all session speakers

6:15-9:30pm: Cocktail reception & welcome dinner for in-person attendees.
6:00-7:00pm: Virtual networking roundtable session #1

**Day 2**

In-person Attendees: 8:00-9:00am ET: Breakfast
Virtual Attendees: 8:00-9:00am ET: Virtual networking roundtable session #2

9:00-1pm ET: SESSION 3 - Clinical trial readiness: outcomes and inclusion
Chair: Adam Boxer MD, PhD, UCSF
- **9:00**: Session opening/welcome – Adam Boxer MD, PhD, UCSF

- **9:05 - 30m**: Measuring what matters: Outcome measures for FTLD
  10m – Shana Dodge PhD, AFTD
  10m – Brad Boeve MD, Mayo Clinic
  10m – Q&A
9:35 - 20m: Equity and inclusion in FTD care and research: gaps and opportunities
Monica Rivera-Mindt PhD, ABPP, Fordham University

9:55 - 30m: Progress in PSP – advances in clinical trials
10m – Adam Boxer MD, PhD, UCSF
10m – Michael Gold MS, MD, Abbvie
10m - Q&A moderated by Kristophe Diaz PhD, CurePSP

10:25 - 15m: Break

10:40 - 20m: Lessons learned during COVID-19: adoption of remote tools
Tim Peters-Strickland MD, PPD Inc

11:00 - 10m: Research readiness – Perspectives from the FTD Insights Survey
Dianna Wheaton MS, PhD, CHES, FTD Disorders Registry

11:10 - 60m: Panel discussion – measuring what matters accurately, precisely, and reliably
Moderators (1 to facilitate virtual comments, 1 live)
Primary moderator – Adam Boxer MD, PhD UCSF
Secondary moderator – Penny Dacks PhD, AFTD
Panelists –
- Brad Boeve MD, Mayo Clinic
- Dr. Monica Rivera-Mindt PhD, ABPP, Fordham University
- Tim Peters-Strickland MD, PPD Inc.
- Carole Ho MD, Denali Therapeutics
- Dianna Wheaton MS, PhD, CHES, FTD Disorders Registry
- Billy Dunn MD, FDA Office of Neuroscience
- Michelle Campbell PhD, FDA Office of Neuroscience

12:10pm ET: Looking to the Future: Wrap up discussion
Primary moderator – Penny Dacks PhD, AFTD
Secondary moderator – Debra Niehoff PhD, AFTD
Panelists –
- Adam Boxer MD, PhD UCSF
- Michael Gold MS, MD, Abbvie
- Howard Feldman MD FRCP(C), UCSD
- Billy Dunn MD, FDA Office of Neuroscience

1pm ET: Adjourn