FTD Treatment Study Group 2021
Innovations and Progress in Clinical Trial Design for FTD
December 8 & 9, 2021

Participants welcomed either at the Doubletree by Hilton Silver Spring, MD or online

Draft agenda as of 11.08.21

Day 1

7:30-8:40am ET: Breakfast

9:00am ET: Welcome
   Susan Dickinson MSGC, AFTD
   Penny Dacks PhD, AFTD

9:15-12:45pm ET: SESSION 1, The Therapeutic Landscape for FTD
Chair: Howard Feldman MD FCRP, UCSD

   • 9:15 - 5m: Welcome – Opening remarks
   • 9:20 - 20m: Keynote: Targeting familial disease: lessons learned
     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
     • Vissia Viglietta MD, PhD, WaveLife Sciences
   • 10:40 - 15m: Break
   • 10:55 - 15m: Landscape of the FTD Biomarkers
     Kaj Blennow MD, 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: Moderated by Kaj Blennow MD, PhD, University of Gothenburg

- 11:35 - 25m: Moderated discussion: Biomarkers for current and future FTD clinical trials
  Moderator (2 needed – 1 to facilitate virtual comments, 1 live)
  Primary discussion moderator – Kaj Blennow MD, PhD, University of Gothenburg
  Secondary discussion moderator – Howard Feldman MD, FCRP, UCSD
  Panelists –
  - Vissia Viglietta MD, 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, FCRP, 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
  TBD

- 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: Clinical trial readiness for rare diseases
  TBD

- 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)
  TBD

• 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

• 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 – AFTD scientific staff
  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

- **10:15 - 15m:** Break

- **10:30 - 20m: Lessons learned during COVID-19: adoption of remote tools**
  Tim Peters-Strickland MD, PPD Inc

- **10:50 - 10m: Research readiness – Perspectives from the FTD Insights Survey**
  Dianna Wheaton MS, PhD, CHES, FTD Disorders Registry

- **11:00-60m: 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
  - TBD

**12:00pm ET: Wrap-up**

**1pm ET: Adjourn**