The Association for Frontotemporal Degeneration
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2700 Horizon Drive, Suite 120
King of Prussia, PA 19406
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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**

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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)**

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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

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- **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**