Thursday, March 8

8:00-11:00 Registration opens

9am – 11am FTD Clinical Trials - Current Landscape
Session Chair: Howard Feldman, UC San Diego

1. **FTD: From Clinico-Pathology to Diagnosis** – Brad Dickerson, Massachusetts General Hospital
   *Fundamentals of FTLD disorders, how to define target populations for clinical trials, can genetic syndromes inform on sporadic FTD*

2. **Autosomal Dominant FTD and Developing Drug Targets** – Brad Boeve, Mayo Clinic Rochester
   *Imaging and fluid biofluids data for MAPT, PGN, C9ORF72, longitudinal imaging as outcome measure*

3. **FTD Clinical Trials – Lessons learned from phase I-III trials** - Adam Boxer, UC San Francisco
   *Landscape of FTLD trials to date, ARTFL & LEFFTDS expert clinical research networks for FTLD trials*

4. **ALS, new therapies, small population trials** – Sabrina Paganoni, Massachusetts General Hospital
   *25 years of ALS trials, emerging biomarkers, common themes and challenges with FTD trial methodology*

11am -12pm Expert panel discussion (speakers + Carole Ho – Denali, Howard Fillit, ADDF, Billy Dunn - FDA ) –
   *critical analysis of trials, sharing natural history data to advance common goals, advances in FTD applied to diseases with overlapping pathologies*

12:00-1:00 LUNCH

1:15pm-4:15pm New Approaches to FTD Outcome Measures
Session Chair: Adam Boxer, UCSF

1. **Developing meaningful clinical endpoints** – Mario Masellis, Sunnybrook Hospital, Toronto
   *Phenotypic complexity, genetic vs sporadic disease, at risk vs symptomatic, limitations of current existing measures*

2. **MR imaging and individualized endpoints** – Howard Rosen, UC San Francisco
   *Short term imaging series to develop optimal individualized endpoints based on multi-modal data, predicting clinical syndromes and subject stratification*

3. **Fluid biomarkers** – Holly Soares, AbbVie
   *Blood-based and CSF biomarkers, pre-analyte standardization, diagnostic vs progression markers*

4. **Alternative study designs, new tools for neurology trials** – Ray Dorsey, U Rochester
   *Basket trials, platform trials, disease modeling, improving R&D productivity*

3:15-3:30 Break

5. **Therapeutic approaches to autosomal dominant FTD syndromes** – Michael Panzara, WaveLife Sciences
   *A roadmap to C9 therapy, challenges of endpoint selection*

6. **Tau therapies, FTD as a proving ground** – Michael Irizarry, Eli Lilly
   *Targeting at-risk populations, FTDP-17 as prototype prevention study, common pathways in movement disorders*
4:30 pm–5:30 pm  Expert panel discussion (speakers + Robert Paul - Alector, Ajay Verma - United Neuroscience, Robert Schuck - FDA, Billy Dunn - FDA) – qualification of clinical outcome assessments and complex disease heterogeneity, genetic modifiers, are common endpoints possible

6pm -7pm  Reception
7pm – 9pm  Dinner

Friday March 9

Registration opens 7:30am

7-7:45am  BREAKFAST

8am-10am  Clinical Trials in the Age of Big Data
Session Chair: Michael Gold, AbbVie

1. **Algorithms and individualized assessments** – Ken Rockwood, DGI Clinical
   *Decision making tools for clinically meaningful data collection, goal attainment scaling, patient reported outcomes*

2. **Recruitment/retention strategies for rare diseases** – Horacio Plotkin, PPD
   *Lessons from other orphan and ultra-orphan diseases, the value of registries and natural histories*

3. **Using integrated databases** - Steve Arneric, Critical Path Institute, CAMD
   *Common principles and data sharing initiatives from AD world*

4. **Digital phenotyping** – William Marks, Verily Life Science
   *Data curation and data sharing, a cloud-based solution*

10-11 am  Expert panel discussion (speakers + Rodney Pearlman - Bluefield Project, Marg Sutherland, NIH, Billy Dunn - FDA) - setting industry comfort level on sharing proprietary data, building a database to support efficiencies in trials

11-11:15  BREAK


11:30-12:30  Designing an FTLD Trial - FTD CLINICAL RESEARCH NETWORKS PANEL (ARTFL, LEFFTDS, GENFI + AFTD MAC) – Adam Boxer, Brad Boeve, Howard Rosen, Mario Masellis, Brad Dickerson, Murray Grossman, Edward Huey, Chiadi Onyike – A straw man model to support development of targeted therapies

12:30-1pm  Wrap up – Session Chairs and AFTD