The Association for Frontotemporal Degeneration
Opening the gateway to help and a cure

FTSG 2018 – Clinical Trials Methodology and Outcome Measures for FTD
Washington Marriott at Metro Center
March 8-9, 2018
DRAFT AGENDA- 2-9-18

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**

11:15-11:30 **Targeting therapies for low-frequency molecular subtypes –** Robert Schuck, FDA-CDER

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