American Institute of Architects, Washington DC
March 21-22, 2019

2019 Research Roundtable for Epilepsy
Efficient Trials in Epilepsy

Thursday, March 21, 2019

12:00 PM **Lunch and Registration** *(AIA Social Gallery)*

1:00 PM **Meeting Welcome and Updates** *(AIA Boardroom)* *Mr. Phil Gattone and Dr. Brandy Fureman*

- 1:05 PM – Meeting goals and deliverables *Dr. Nathan Fountain and Dr. Jacqueline French*
- 1:15 PM – Recap of 2018 RRE and new developments *Drs. Fountain and French*
- 1:25 PM – European Medicines Agency (EMA) update *Prof. Michel Baulac*
- 1:35 PM – Caregiver Panel: Caregiver perception of risk versus benefit, family burden and other pragmatic considerations for trial design
  Ms. Vanessa Vogel-Farley (Dup15q Alliance); Ms. Paige Nues (Rett Syndrome Foundation); Ms. Kim Nye (TESS Research Foundation); Ms. Megan Roberts (KCNQ2 Cure Alliance)

2:15 PM **Session I, PART A: Proof of concept for new therapies**

- 2:05 PM – Talk 1: What is a useful pharmacodynamic signal?
  Dr. Alexander Rotenberg and Dr. Jacqueline French
- 2:35 PM – Talk 2: Debate: Open label studies for proof of concept: Do they tell us anything?
  Dr. Nathan Fountain and Dr. Roger Porter
- 2:55 PM – Discussion

3:10 PM **BREAK**

3:20 PM **Session I, Sub-session: Seizure counting in trials**

- 3:20 PM - Talk 3: Scenarios of seizure counting *Dr. Dennis Dlugos*
- 3:30 PM – Talk 4: Optimizing seizure counting on EEG *Dr. Sudha Kessler*
- 3:50 PM – Talk 5: EEG modalities for seizure counting *Dr. Dean Freestone*
- 4:05 PM – Open Discussion on alternative endpoints
- 4:35 PM – FDA requirements for diagnostic seizure counting devices *Dr. Jay Gupta*

4:45 PM **Session I, Sub-session: Approach to safety characterization in short/small trials**

- 4:45 PM - Talk 6: How to adequately characterize the safety profile with shorter or smaller trial
  Dr. David Blum
- 5:05 PM - Open Discussion

5:30 PM **Adjourn Day 1**

6:15 PM **Reception** *(at W Hotel)*
7:00 PM **Dinner** *(at W Hotel)*

Friday, March 22, 2019

7:45 AM **Breakfast** *(AIA Social Gallery)*

8:30 AM **Session I, PART B: Definitively evaluating effectiveness in very small populations** *(AIA Boardroom)*

- 8:30 AM – Keynote: Reflections on the nusinersen development program
  Dr. Billy Dunn and Dr. Alfred Sandrock
- 9:00 AM – Panel: Pragmatic considerations for trials in ultra-rare diseases
  Dr. Dimitrios Arkilo (Takeda); Dr. Walter Kaufmann (Anavex); Dr. Gail Farfel (Zogenix); Dr. Y. Paul Goldberg (Ionis); Dr. Bruno Flamion (Indorsia)
- 9:25 AM – Open Discussion

10:00 AM **NIH anti-epileptogenesis workshop summary & discussion** *Dr. Adam Hartman*

10:15 AM **BREAK**

10:25 AM **Session II: Improving efficiency of the standard design**

PART A: Study designs and time to event

- 10:25 AM – Talk 1: Pros and cons of new designs compared to standards *Dr. Jacqueline French*
- Time to event trials
  - 10:40 AM – Talk 2: New design for infantile seizures from PERC/ILAE
    Dr. Renée Shellhaas
  - 10:55 AM – Talk 3: UCB Post-hoc analysis using infant trial time to event concept
    Dr. Ali Bozorg
  - 11:05 AM – Talk 4: Time to event in practice *Dr. Konrad Werhahn*

11:15 AM **Open Discussion**

12:00 PM **LUNCH**

1:00 PM **PART B: Optimal dosing and duration**

- 1:00 PM – Talk 1: What is the minimal Study duration / Seizure frequency needed to demonstrate efficacy *Dr. Ed Whalen*
- 1:40 PM – Talk 2: Dosing: Phase 3 study designs and Flexible dosing *Dr. Roger Porter*
- 1:55 PM – Talk 3: What is the best Phase 2 study design to determine the optimal dose
  Dr. John Messenheimer

2:10 PM **Open Discussion**

3:00 PM **Wrap up and Conclusions** *Drs. Fountain and French*

3:10 PM **Adjourn**