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