Phase II Experiences in Current Alzheimer's Disease Trials
An FDA/Alzheimer's Disease Allies Meeting

Hilton Executive Meeting Center
1750 Rockville Pike Rockville, Maryland
December 9, 2011

7:30– 8:00 a.m. **Registration and Breakfast**

WELCOME

8:15-8:30 a.m. **Introduction by ACT-AD and Co-hosts**
Dan Perry, ACT-AD
George Vradenburg, LEAD
Tim Armour, Cure Alzheimer's Fund

PRESENTATIONS

Designing a Phase II Trial for Alzheimer's disease

8:30-9:05 a.m. Solanezumab
Eric Siemers, Eli Lilly

9:10-9:40 a.m. Avagacestat (BMS-708163)
Alan Lipschitz, BMS

9:45-10:15 a.m. IVIG
David Gelmont, Baxter

10:20-10:50 a.m. MABT 5102A
Carole Ho, Genentech

Gantenerumab
Susanne Ostrowitzki, Roche

Questions for presenter to address:

- *How did you decide what population to select?*
- *Traditionally one does not expect to show efficacy in phase II. Did you choose an efficacy signal(s) for phase II? If so, which did you choose for phase II and why?*

- *What was your approach to incorporation of biomarkers and why?*
- *Does your trial include endpoints to support disease modification?*
- *Are there limitations that still exist in your study?*
- *For compounds that have moved beyond phase II, has science advanced to provide information that would have been helpful to you in designing your study? What might you have done differently?*

10:55-11:25a.m. **Current Thinking on Adaptive Trial Designs**
Michael Krams, Janssen AI

11:30 -11:50 a.m. **Q&A**

PANEL

11:55 a.m.-12:55 p.m. **Examining Phase II Trial Design Issues**

Discussion Leader: Rachelle Doody, Baylor College of Medicine
Panelists: Reisa Sperling, Harvard Brigham and Women's
Paul Aisen, UCSD
Harald Hampel, University of Frankfurt
Russell Katz, U.S. Food and Drug Administration

Suggested questions for panel to address:

- *What are the best strategies for improving identification of study subjects in the absence of a validated antemortem diagnostic test?*
- *Is there a rationale for combining patients at different stages of presumed AD dementia in clinical trials?*
- *What are the key differences that need to be considered when designing a trial for interventions targeting patients at different stages of dementia?*
- *What are the most promising candidates for and uses of biomarkers as dynamic indicators of disease progression?*
- *At what stages of Alzheimer's disease can one establish the validity of treatment effect on a biomarker and demonstrate its likeliness to predict clinical effects?*
- *Is the field close to demonstrating that an effect on a biomarker or even an array of biomarkers by itself translates into a useful drug clinically?*
- *How can the time of therapeutic intervention be better matched with the compound's mechanism of action at different stages of Alzheimer's disease?*

1:00– 1:40 p.m. **Q&A**

CLOSING

1:45-2:00 pm **Concluding remarks**
Dan Perry