Over the last few decades results from Phase III Parkinson's trials have been uniformly negative despite ever increasing knowledge about Parkinson's biology and progressively solid premises for moving agents to large trials. The community, therefore, feels that a reassessment of our approach to disease modification in Parkinson's is needed. This workshop aims to address this need by providing a forum to exchange experiences, learn from past mistakes, discuss ideas about the extent of data needed prior to clinical testing and rigor that needs to be observed, and, hopefully, generate insight about changes the field needs to implement.

AGENDA DAY 1

8:00 a.m. **Welcome and Opening Remarks**
Walter Koroshetz, Director, NINDS
Codrin Lungu and Beth-Anne Sieber, Program Directors, NINDS

SESSION I: EXPERIENCE TO DATE

8:30 a.m. Karl Kieburtz, Director, Clinical & Translational Science Institute, University of Rochester
History of disease modification efforts in PD

Lessons from Specific Trials

8:50 a.m. Debra Babcock, Program Director, Division of Neuroscience, NINDS
NET-PD

9:05 a.m. Michael Schwarzschild, Professor of Neurology, Harvard Medical School
SURE-PD

9:20 a.m. Tanya Simuni, Chief of Movement Disorders, Dept. of Neurology, Northwestern Medicine
STEADY-PD

9:35 a.m. Audience Q&A

Lessons from Other Fields

9:55 a.m. Robert Fox, Vice Chair for Research, Neurological Institute, The Cleveland Clinic
Multiple Sclerosis

10:15 a.m. TBD
Spinal Muscular Atrophy

10:35 a.m. Audience Q&A

10:55 a.m. ***Break***

SESSION II: INCREASING THE CHANCES OF IDENTIFYING EFFECTIVE TREATMENTS

11:10 a.m. Ted Dawson, Professor of Neurodegenerative Diseases, Johns Hopkins University
Basic science

11:30 a.m. Amir Tamiz, Director, Division of Translational Research, NINDS
Rigor and target validation

11:50 a.m. Andrew West, Consulting Professor, Dept. of Pharmacology and Cancer Biology, Duke University
Biomarkers: patient selection, disease progression, target engagement, and proof of principle

12:10 p.m. Christopher Coffey, Director, Clinical Trials Statistical Data Management Center, University of Iowa
Clinical trial readiness and early trial design - population selection, outcome measures, platform design, best relevance to pathophysiology

12:30 p.m. Audience Q&A

12:50 p.m. ***Lunch***

SESSION II: CONTINUED

Agent Selection for Phase II Studies

1:50 p.m. Marie-Françoise Chesselet, Emerita Distinguished Professor of Neurology and Neurobiology, UCLA
What data from animal models is necessary and/or useful?

2:05 p.m. Kevin Biglan, Medical Fellow at Eli Lilly; Adjunct Clinical Prof. of Neurology, University of Rochester
Interpreting epidemiologic intervention effect data on PD risk

2:20 p.m. Patrik Brundin, Associate Director of Research, Van Andel Research Institute
Linked Clinical Trials (LCT) initiative approach

2:35 p.m. Brian Fiske, Senior Vice President, Research Programs, Michael J. Fox Foundation
Michael J. Fox Foundation approach

2:50 p.m. Clinton Wright, Director, Division of Clinical Research, NINDS
NINDS approach

3:05 p.m. James Beck, Senior Vice President, Chief Scientific Officer, Parkinson's Foundation
Parkinson's Foundation approach

3:20 p.m. Jesse Cedarbaum, Head, Coeruleus Clinical Sciences
Industry approach

3:35 p.m. Audience Q&A

4:00 p.m. ***Adjourn***

AGENDA DAY 2

SESSION III: CONSIDERATIONS OF IMPORTANCE FOR PHASE III STUDIES

8:00 a.m. Howard Federoff, CEO, Aspen Neuroscience; Professor of Neurology, UC Irvine
Evidence of target engagement or proof of principle from Phase II

8:20 a.m. Eric Macklin, Biostatistician, Biostatistics Center, Massachusetts General Hospital
How to design Phase III trials

8:40 a.m. Bernard Ravina, Chief Medical Officer, Praxis Medicines
NIH vs Industry Phase III trials

9:00 a.m. Alberto Espay, Endowed Chair, Center for PD and Movement Disorders, University of Cincinnati
Population selection. One disease vs. many.

9:20 a.m. Rachel Saunders-Pullman, Associate Professor of Neurology, Mt. Sinai Beth Israel
Genotypes and phenotypes

9:40 a.m. Ray Dorsey, Director, Center for Health and Technology, University of Rochester
New tools and technologies to be leveraged

10:00 a.m. **Example of Synergy Opportunities: Nilotinib and other c-Abl inhibitors**

Ted Dawson, Professor of Neurodegenerative Diseases, Johns Hopkins University

Andrew Goldfine, Medical Director, Sun Pharma

Fernando Pagan, Vice Chairman, Dept. of Neurology, Georgetown University

Tanya Simuni, Chief of Movement Disorders, Dept. of Neurology, Northwestern Medicine

Milton Werner, Founder, President, & CEO, Inhibikase

10:30 a.m. Audience Q&A

10:55 a.m. **SESSION IV: NEXT STEPS DISCUSSION**

I. **Developing a Collaborative Roadmap for Future Disease Modifying Trials**
II. **Articulating a Standard Structure Approach to Drug Selection for Phase II Studies**
III. **Building a Framework for Moving Candidates from Phase II to Phase III Testing**

11:40 a.m. **CONCLUSIONS AND ACTION ITEMS**