



Agenda

January 6, 2014 (Day 1)

8:00 a.m. **Welcome and Introduction**

Story C. Landis, PhD
Director, NINDS

8:15 a.m. **Meeting Goals**

Thomas J. Montine, MD, PhD
Alvord Professor and Chair
Department of Pathology, University of Washington

8:30 a.m. **Bringing Emerging Science to People with Parkinson's Disease through Clinical Research**

Session Chairs: Randall Bateman, MD, and Andrew Siderowf, MD
NINDS Liaisons: Wendy Galpern, MD, PhD, and Walter Koroshetz, MD

Introduction

Randall Bateman, MD
Knight Distinguished Professor of Neurology
Washington University School of Medicine

Andrew Siderowf, MD
Medical Director
Avid Radiopharmaceuticals

8:40 a.m. **Opportunities to Advance Symptomatic Treatment of Motor and Non-motor Features**

Presenter: Werner Poewe, MD
Director, Department of Neurology
Innsbruck Medical University

- 8:55 a.m. Panel Discussion of Recommendations
- Moderator:* Andrew Siderowf, MD
Panelists: Karen Marder, MD, MPH; Werner Poewe, MD; Bernard Ravina, MD; Lisa Shulman, MD; Philip Starr, MD, PhD*; and Matthew Stern, MD.
- 9:15 a.m. **Opportunities for Innovation in Trial Design**
- Presenter:* Christopher Coffey, PhD
Professor, Department of Biostatistics
University of Iowa
- 9:30 a.m. Panel Discussion of Recommendations
- Moderator:* Randall Bateman, MD
Panelists: Christopher Coffey, PhD; Jordan Elm, PhD; Nicholas Kozauer, MD*; Bernard Ravina, MD; Ira Shoulson, MD*; and Philip Starr, MD, PhD*.
- 9:50 a.m. Break
- 10:20 a.m. **Opportunities to Improve Outcome Measures in PD Research**
- Presenter:* Lisa M. Shulman, MD
Professor of Neurology
University of Maryland School of Medicine
- 10:35 a.m. Panel Discussion of Recommendations
- Moderator:* Andrew Siderowf, MD
Panelists: Jordan Elm, PhD; Nicholas Kozauer, MD*; Lisa Shulman, MD; and Caroline Tanner, MD, PhD.
- 10:55 a.m. **Opportunities for Understanding and Addressing Disease Progression**
- Presenter:* Caroline Tanner, MD, PhD
Director, Clinical Research
The Parkinson's Institute
- 11:10 a.m. Panel Discussion of Recommendations
- Moderator:* Randall Bateman, MD
Panelists: Christopher Coffey, PhD; Karen Marder, MD, MPH; Werner Poewe, MD; Ira Shoulson, MD*; Matthew Stern, MD; and Caroline Tanner, MD, PhD.

11:30 a.m. **Panel Discussion: Priority Ranking of Clinical Research Recommendations**

Moderators: Randall Bateman, MD and Andrew Siderowf, MD

Panelists: Christopher Coffey, PhD; Jordan Elm, PhD; Karen Marder, MD, MPH; Werner Poewe, MD; Bernard Ravina, MD; Lisa Shulman, MD; Matthew Stern, MD; and Caroline Tanner, MD, PhD.

12:00 p.m. Lunch

1:00 p.m. **Building a Translational Pipeline for Parkinson's Disease Therapeutics**

Session Chairs: John Dunlop, PhD, and Steven Finkbeiner, MD, PhD

NINDS Liaisons: Margaret Sutherland, PhD, and Hao Wang, PhD

Introduction

John Dunlop, PhD
Vice President, Neuroscience
AstraZeneca

Steven Finkbeiner, MD, PhD
Associate Director & Senior Investigator
Gladstone Institute of Neurological Disease
University of California San Francisco

1:10 p.m. **Patient/Disease Stratification**

Presenter: John Dunlop, PhD
Vice President, Neuroscience
AstraZeneca

1:25 p.m. **Panel Discussion of Recommendations**

Moderator: John Dunlop, PhD

Panelists: Rudi Balling, PhD; Steven Finkbeiner, MD, PhD; John Hardy, PhD; and Dean Jones, PhD.

1:45 p.m. **Target Engagement and Efficacy**

Presenter: Dimitri Krainc, MD, PhD
Professor and Chairman, Department of Neurology
Northwestern University

- 2:00 p.m. Panel Discussion of Recommendations
Moderator: Steven Finkbeiner, MD, PhD
Panelists: John Dunlop, PhD; John Hardy, PhD; Hartmuth Kolb, PhD*; Dimitri Krainc, MD, PhD; Kalpana Merchant, PhD*; and Richard Youle, PhD.
- 2:20 p.m. **Modeling Parkinson's Disease Pathways: Forward and Reverse Translation**
- 2:20 p.m. *Presenter:* Michael Lee, PhD
Professor, Department of Neuroscience
University of Minnesota
- 2:30 p.m. *Presenter:* Clive Svendsen, PhD
Director, Regenerative Medicine Institute
Cedars Sinai Health System, Los Angeles
- 2:40 p.m. Panel Discussion of Recommendations
Moderator: Steven Finkbeiner, MD, PhD
Panelists: Wade Harper, PhD; Michael Lee, PhD; Clive Svendsen, PhD; and Richard Youle, PhD.
- 3:00 p.m. Break
- 3:20 p.m. **Target Identification and Validation: Criteria for Advancement**
Presenter: Richard Youle, PhD
Senior Investigator and Chair, Biochemistry Branch
NINDS, NIH
- 3:30 p.m. *Presenter:* Wade Harper, PhD
Professor of Cell Biology
Harvard Medical School
- 3:40 p.m. Panel Discussion of Recommendations
Moderator: Wade Harper, PhD
Panelists: Rudi Balling, PhD; John Hardy, PhD; Dean Jones, PhD; and Dimitri Krainc, MD, PhD.
- 4:00 p.m. **Panel Discussion: Priority Ranking of Translational Research Recommendations**
Moderator: John Dunlop, PhD

Panelists: Rudi Balling, PhD; Steven Finkbeiner, MD, PhD; John Hardy, PhD; Wade Harper, PhD; Dean Jones, PhD; Dimitri Krainc, MD, PhD; Michael Lee, PhD; Clive Svendsen, PhD; and Richard Youle, PhD.

4:30 p.m. **Parkinson's Disease Research Evaluation: A Multi-Stakeholder Perspective**

Session Chair: Amy Comstock Rick, JD
NINDS Liaison: Beth-Anne Sieber, PhD

Introduction

Amy Comstock Rick, JD
Chief Executive Officer, Parkinson's Action Network

4:35 p.m. **Research Evaluation: An AAMC Initiative**

Ann C. Bonham, PhD
Chief Scientific Officer, Association of American Medical Colleges

4:45p.m. **Panel Discussion**

Moderator: Amy Comstock Rick, JD
Panelists: Ann C. Bonham, PhD; Story C. Landis, PhD; Bernard Ravina, MD; Todd Sherer, PhD; and David Sulzer, PhD.

January 7, 2014 (Day 2)

9:00 a.m. **Parkinson's Disease Biology: Moving towards Innovative Treatments**

Session Chairs: Virginia M-Y Lee, PhD, and Harry Orr, PhD
NINDS Liaisons: Katrina Gwinn, MD, and Kip Ludwig, PhD

Introduction

Virginia M-Y Lee, PhD
Director, Center for Neurodegenerative Disease Research
University of Pennsylvania Perelman School of Medicine

Harry T. Orr, PhD
Professor of Pathology
University of Minnesota

9:10 a.m. **Pathways and Signaling**

Presenter: Harry T. Orr, PhD
Professor of Pathology
University of Minnesota

- 9:25 a.m. Panel Discussion of Recommendations
- Moderator:* Mel Feany, MD, PhD
Panelists: Ted M. Dawson, MD, PhD; Harry T. Orr, PhD; and Andrew Singleton, PhD.
- 9:55 a.m. Break
- 10:25 a.m. **Protein Processing, Aggregation, and Spread**
- Presenter:* Virginia M-Y Lee, PhD
Director, Center for Neurodegenerative Disease Research
University of Pennsylvania Perelman School of Medicine
- 10:40 a.m. Panel Discussion of Recommendations
- Moderator:* Robert Edwards, MD
Panelists: Hilal Lashuel, PhD; Virginia M-Y Lee, PhD; and J. Paul Taylor, MD, PhD.
- 11:10 a.m. **Circuit Modification**
- Presenter:* Warren M. Grill, PhD
Professor of Biomedical Engineering
Duke University
- 11:25 a.m. Panel Discussion of Recommendations
- Moderator:* Kendall Lee, MD, PhD
Panelists: Ann Graybiel, PhD; Warren Grill, PhD; and Mark Schnitzer, PhD*.
- 11:55 a.m. **Panel Discussion: Priority Ranking of Basic Research Recommendations**
- Moderators:* Virginia M-Y Lee, PhD, and Harry Orr, PhD.
Panelists: Ted Dawson, MD, PhD; Robert Edwards, MD; Mel Feany, MD, PhD; Ann Graybiel, PhD; Warren Grill, PhD; Hilal Lashuel, PhD; Kendall Lee, MD, PhD; Andrew Singleton, PhD; and J. Paul Taylor, MD, PhD.
- 12:30 p.m. Lunch
- 1:30 p.m. Public Comment
- Moderators:* Thomas J. Montine, MD, PhD, and Beth-Anne Sieber, PhD
- 2:30 p.m. Defining Priorities for Parkinson's Disease Research

Moderator: Thomas J. Montine, MD, PhD

Panelists: Randall Bateman, MD; John Dunlop, PhD; Steven Finkbeiner, MD, PhD; Virginia M-Y Lee, PhD; Harry Orr, PhD; and Andrew Siderowf, MD.

4:00 p.m. **Closing Comments**

Story C. Landis, PhD
Director, NINDS

4:30 p.m. Adjourn

*Panel members who participated in recommendation development, but are unable to attend this conference.