



Despite substantial public and private interest in developing therapeutics for the Parkinson's disease (PD) community, there persists separation between disease understanding and development of drugs to target these processes in Parkinson's patients. The NINDS is convening a hybrid workshop with the overarching goal to (1) delineate what the crucial elements of those gaps are and (2) determine how to close those gaps as a community of stakeholders, including academic researchers, industry partners, non-profit organizations, and people with lived experience of Parkinson's Disease.

All times are Eastern Standard Time

April 23, 2024 (Day 1)

Welcome and Opening remarks

9:00 am NINDS Welcome

Walter J. Koroshetz, MD, Director, National Institute of Neurological Disorders and Stroke

9:10 am Engaging the lived experience perspective on PD Therapeutics

Rebekah Corlew, PhD, Lived Experience Engagement Strategist, National Institute of Neurological Disorders and Stroke

Keynote Address

9:20 am Landscape of PD therapeutics development

Workshop Co-Chairs welcome and goals for the 2 days

Alice Chen-Plotkin, MD, University of Pennsylvania

Bill Martin, PhD, Johnson & Johnson Innovative Medicine

10:00 am Break (5 minutes)

Session 1: PD Heterogeneity

Moderator: Karl Kieburtz, MD, MPH, University of Rochester School of Medicine and Dentistry

10:05 am Motor Heterogeneity in Parkinson's Disease: Challenges to Therapeutic Developments

Anthony Lang, MD, FRCPC, University of Toronto

10:20 am Heterogeneity of Non-Motor Symptoms across Lewy Body Diseases

Kathleen Poston, MD, MS, Stanford University

10:35 am Q & A discussion

11:05 am Break (10 minutes)

Session 2: Target validation

Moderator: Alice Chen-Plotkin, MD, University of Pennsylvania

11:15 am Evolving Arcs toward Disease-Modifying Therapy for Parkinson's

Michael Schwarzschild, MD, PhD, Massachusetts General Hospital

11:30 am Targeting LRRK2: Why, Who, Where and When

Mark R. Cookson, PhD, National Institute on Aging

11:45 am An Industry Perspective on Target Validation For Neurologic Diseases

David Stone, PhD, Cerevel Therapeutics, LLC

12:00 pm Q & A discussion

12:30 pm Lunch (60 minutes)

Session 3: Appropriate tools to enable therapeutics development

Moderator Shalini Padmanabhan, PhD, Michael J. Fox Foundation

1:30 pm Teeing Up a Discussion about Appropriate Tools to Enable Therapeutics Development

Patrik Brundin, MD, PhD, Roche

1:45 pm Preclinical Tools to Advance Parkinson Disease Therapeutics: What They Can and Cannot Do

Laura Volpicelli-Daley, PhD, University of Alabama at Birmingham

2:00 pm Q & A discussion

2:30 pm Break (10 minutes)



Session 4: Biomarker use

Moderator: Danielle Graham, PhD, Biogen

- 2:40 pm AI-Enabled Biomarkers: A Window into Parkinson's Progression, Detection, and Treatment Response**
Dina Katabi, PhD, Massachusetts Institute of Technology
- 2:55 pm Identification of Proteomic Targets and Biomarkers: The Mapping Proteomics to Parkinson's Disease Project (MAP2PD)**
Laura Winchester, DPhil, University of Oxford
- 3:10 pm Understanding Biomarker Parameters to Enable Clinical Trials in Parkinson's Disease**
Danielle Graham, PhD, Biogen
- 3:25 pm Q & A discussion**
- 3:55 pm Break (15 minutes)**

Breakout Session Day 1

- 4:10 pm What is missing that is holding back therapeutics development?**
- 4:55 pm Break (5 minutes)**

End of Day 1

- 5:00 pm** Day 1 wrap-up
- 5:10 pm** Adjourn for the day

April 24, 2024 (Day 2)

Welcome and Day 2 Opening remarks

8:45 am Alice Chen-Plotkin, MD and Bill Martin, PhD

Breakout Session Reports from Day 1

- 8:55 am Reports from Breakout sessions and Q & A**
Breakout reports by the breakout session leaders followed by general discussion (20 minutes)
- 10:00 am Break (10 minutes)**

Session 5: Lessons from PD adjacent communities

Moderator: Amir Tamiz, PhD, Director, Division of Translational Research, National Institute of Neurological Disorders and Stroke

- 10:10 am Disease-Modifying Therapies: Lessons from Alzheimer's Disease Drug Development**
Jeffrey Cummings, MD, ScD, University of Nevada, Las Vegas
- 10:25 am RNA-Target Therapies for Neurodegenerative Disease**
Timothy M. Miller, MD, PhD, Washington University School of Medicine
- 10:40 am Q & A discussion**

11:10 am Break (10 minutes)

Session 6: Best practices for advancing development

Moderator: Bill Martin, PhD, Johnson & Johnson Innovative Medicine

- 11:20 am Addressing the Academia-Industry Gap to Advance Discoveries to the Clinic**
Opher Kornfeld, PhD, SPARK NS
- 11:35 am LRRK2 inhibitors: From Basic Biology to the Clinic**
Anastasia Henry, PhD, Denali Therapeutics
- 11:50 am Opportunities and Challenges in Parkinson's Disease Clinical Development using the Example of the SCD Inhibitor YTX-7739**
Fiona Elwood, PhD, Johnson & Johnson Innovative Medicine
- 12:05 pm Q & A discussion**

End of Day 2

- 12:35 pm** Day 2 wrap-up
- 12:45 pm** Adjourn / Lunch (75 minutes)

Optional Breakout Session Day 2

- 2:00 pm Parallel breakout sessions**
- 1) What to know about IP and patents, with Lauren Nguyen-Antczak, PhD, National Cancer Institute and Susan Ano, PhD, NINDS
 - 2) Forming partnerships with industry, with Bill Martin, Patrik Brundin, and John Sullivan, PhD, Entrepreneur in Residence, SEED, NIH
 - 3) Starting a company, with Opher Kornfeld and Leon Chen, PhD, The Column Group
- 3:00 pm** Adjourn