

2026 Parkinson's Disease Therapeutics Conference

October 14 – 15, 2026
New York City

Table of Contents

03	Welcome
05	Day 1 Agenda
08	Day 2 Agenda
11	Sponsors
16	Abstracts



2025 PARKINSON'S DISEASE
THERAPEUTICS CONFERENCE

Martin Handfield
Neuropacs Corp.

Welcome

Welcome to the 18th annual Parkinson's Disease Therapeutics Conference. This year's conference comes at an exciting moment for Parkinson's research, as new biological insights and advances in therapeutic development are creating opportunities to approach the disease with greater precision than ever before. At The Michael J. Fox Foundation (MJFF), we are proud to bring this community together to exchange ideas, explore emerging science and accelerate the path from discovery to meaningful advances for people with Parkinson's.

Today, the Parkinson's research landscape is advancing at an unprecedented pace. Our ever-growing access to high-quality datasets and biosamples is revealing the complexity of Parkinson's biology and helping to deepen our understanding of why the disease differs from person to person.

These insights are transforming how the field approaches Parkinson's, accelerating a shift toward precision medicine and the ability to deliver the right treatment to the right person at the right time. PPMI's evolution from the Parkinson's Progression Markers Initiative to the Parkinson's Precision Medicine Initiative reflects this ambition and its expanding role in defining Parkinson's biology and driving more targeted therapeutic development.

Against this backdrop of scientific opportunity, MJFF has launched its most ambitious campaign to date: a \$2.5 billion effort to fuel the next era of Parkinson's research by advancing precision medicine, deepening our understanding of disease biology and bringing new therapies closer to patients. This investment will enable the field to pursue breakthroughs with the scale, speed and ambition this moment demands.

In the sessions ahead, you'll hear from colleagues around the world about the latest findings shaping the future of Parkinson's research and therapeutic development. MJFF remains committed to supporting this work with the resources, tools and connections needed to move promising science forward.

We encourage you to engage deeply with your peers and with the Foundation team, sharing ideas, building collaborations and leaving inspired to advance the next generation of Parkinson's therapies.

Sincerely,

A handwritten signature in black ink, appearing to read 'Sohini Chowdhury', with a stylized flourish at the end.

Sohini Chowdhury
Chief Program Officer

About The Michael J. Fox Foundation for Parkinson's Research

As the world's largest nonprofit funder of Parkinson's research, The Michael J. Fox Foundation is dedicated to accelerating a cure for Parkinson's disease and improving therapies for those living with the condition today. The Foundation pursues its goals through an aggressively funded, highly targeted research program coupled with active global engagement of scientists, Parkinson's patients, business leaders, clinical trial participants, donors and volunteers. In addition to funding \$2.5 billion in research to date, the Foundation has fundamentally altered the trajectory of progress toward a cure. Operating at the hub of worldwide Parkinson's research, the Foundation forges groundbreaking collaborations with industry leaders, academic scientists and government research funders; creates a robust open-access data set and biosample library to speed scientific breakthroughs and treatment with its landmark clinical study, PPMI; increases the flow of participants into Parkinson's disease clinical trials with its online tool, Fox Trial Finder; promotes Parkinson's awareness through high-profile advocacy, events and outreach; and coordinates the grassroots involvement of thousands of Team Fox members around the world.

michaeljfox.org

 [michaeljfoxfoundation](https://www.facebook.com/michaeljfoxfoundation)

 [@michaeljfoxorg](https://www.instagram.com/michaeljfoxorg)

 [The Michael J. Fox Foundation for Parkinson's Research](https://www.linkedin.com/company/the-michael-j-fox-foundation-for-parkinsons-research)

The Michael J. Fox Foundation for Parkinson's Research
2026 Parkinson's Disease Therapeutics Conference
October 14, 2026
New York, NY

Conference Program

All presentations are followed by Q&A.

DAY 1

1:00 – 2:00 p.m. **Attendee Arrivals and Networking**

2:10 – 2:40 **MJFF Keynote Presentation**
MARK FRASIER, PhD, The Michael J. Fox Foundation

2:55 – 4:45 **Breakout Rooms** (Attendees Can Choose to Attend Up to 3 Rooms)

2:55 – 3:25 First Room of Your Choice

3:35 – 4:05 Second Room of Your Choice

4:15 – 4:45 Third Room of Your Choice

4:45 – 6:00 **Networking Reception**

ROOM OPTIONS

Room A: Advancing Targets to Therapies

ALLIE VAIANA, PhD, The Michael J. Fox Foundation

GAIA SKIBINSKI, PhD, The Michael J. Fox Foundation

FIONA DUCOTTERD, PhD, University College London

JONATHAN BEHR, PhD, Advisor to The Michael J. Fox Foundation

Explore how MJFF's Targets to Therapies initiative can provide industry with the resources to evaluate emerging PD targets and inform decisions about which opportunities to advance.

Room B: PPMI: Advancing Drug Development in Parkinson's and Related Disorders

CATHERINE KOPIL, PhD, The Michael J. Fox Foundation

KENNETH MAREK, MD, Institute for Neurodegenerative Disorders

TANYA SIMUNI, MD, FAAN, Northwestern University Feinberg School of Medicine

Learn how PPMI data, biomarkers and regulatory insights — alongside the Path to Prevention (P2P) platform trial — can inform more successful clinical trials.

Room C: MJFF Biomarker Toolkits & Resources

ARIANA FARRAND, PhD, The Michael J. Fox Foundation

CHRISTINE SWANSON-FISCHER, PhD, The Michael J. Fox Foundation

KATRINA PAUMIER, PhD, Octave Bioscience

Explore MJFF's biomarker toolkits and resources for measuring key PD biological pathways, and help shape how these drug development tools can better support clinical development.

Room D: Multimodal Data and Cohorts to Enable Precision Medicine

SAMANTHA HUTTEN, PhD, The Michael J. Fox Foundation

CHRIS WHELAN, PhD, Ignition Scientific

KENDALL VAN KEUREN-JENSEN, PhD, TGen

Learn how rapidly expanding multimodal datasets from PPMI, GP2 and other efforts can be leveraged for target discovery, patient stratification, biomarker development and trial design.

Room E: Patient Centered Strategies for Drug Development

MAGGIE KUHL, The Michael J. Fox Foundation

WES KUTLER, The Michael J. Fox Foundation

Learn how incorporating patient insights earlier in therapeutic development can strengthen R&D decisions, and how MJFF can help companies engage the patient community along the way.

Poster Session

DAY 1

National Parkinson's Project: A Federal Framework to Accelerate Progress Against Parkinson's Disease and Related Disorders

DANIELLE MUTONE-SMITH, The Michael J. Fox Foundation

Advancing GBA1 Biomarkers: The GBA1 Industry Biomarker Consortium

FERNANDA CERQUEIRA, PhD, The Michael J. Fox Foundation

Accelerating Parkinson's Disease Research Through Shared Resources

NICOLE POLINSKI, PhD, The Michael J. Fox Foundation

The Michael J. Fox Foundation for Parkinson's Research
2026 Parkinson's Disease Therapeutics Conference
October 15, 2026
New York, NY

Conference Program

All presentations are followed by Q&A.

DAY 2

7:45 – 8:30 a.m.

Arrivals and Breakfast

8:45 – 9:00

MJFF Welcome and Housekeeping

SOHINI CHOWDHURY, The Michael J. Fox Foundation

Session 1: Emerging Therapeutic Strategies in Parkinson's Disease

9:05 – 9:15

Session Introduction From Session Chair

MAGDALENE MORAN, PhD, Topo Therapeutics

9:15 – 10:30

Company Fast Fire Presentations

9:15 – 9:30

Brain Shuttle-enabled Delivery of a TrkB Agonist Antibody for Disease Modification in Parkinson's Disease

PAWEL STOCKI, PhD, Ossianix

9:30 – 9:45

20S Proteasome Activation for the Treatment of Parkinson's Disease

DIOGO FELECIANO, PhD, Booster Therapeutics

9:45 – 10:00

Development of Small-molecule Inhibitors of the Mitochondria Permeability Transition Pore for the Treatment of Parkinson's Disease and ALS

RICHARD RUTTER, PhD, NRG Therapeutics

10:00 – 10:15

Development of a TRPML1 Agonist for GD-PD and Beyond

LEON MURPHY, PhD, Casma Therapeutics

10:15 – 10:30

HER-096: A Subcutaneous CDNF-derived Peptide for Parkinson's disease — Rationale and Phase 1b Results

HENRI HUTTUNEN, PhD, Herantis Pharma Plc

10:40 – 11:15

Panel Discussion

11:15 – 11:45

Networking Break / Poster Viewing

Session 2: Enabling Precision Medicine Approaches in Clinical Development

11:55 a.m. – 12:05 p.m.

Session Introduction From Session Chair
JOHANNES STREFFER, MD, Lundbeck

12:05 – 12:25

The Latest on α -Synuclein Imaging
JAMIE EBERLING, PhD, The Michael J. Fox Foundation

12:25 – 12:45

Detect PD: Proteomic Testing for Development and Progression of Neuronal α -Synuclein Disease

CELINE ROEDER, Queen Mary University of London
JULIA CARRASCO ZANINI SANCHEZ, PhD, Queen Mary University of London

12:45 – 1:05

Polygenic Risk Scores in Neuronal α -Synuclein Disease Stage Progression
MANUELA TAN, PhD, University College London

1:05 – 1:25

Multivariate Latent Disease Progression Modeling in Neuronal α -Synuclein Disease
BARBARA WENDELBERGER, PhD, Berry Consultants

1:30 – 1:40

Closing Remarks and Audience Questions

1:45 – 2:45

Lunch / Networking Break / Poster Viewing

Session 3: “Hot Topics” — The Future of Durable Therapies in Parkinson’s Disease

3:00 – 3:15

Session Introduction From Session Chair
BRIAN FISKE, PhD, The Michael J. Fox Foundation

3:15 – 4:00

Panel Discussion

Moderator: **BRIAN FISKE, PhD**, The Michael J. Fox Foundation

Panelists:

JOOHI JIMENEZ-SHAHED, MD, Mt. Sinai
MAGGIE KUHL, The Michael J. Fox Foundation
PATRIK BRUNDIN, MD, PhD, Roche
RICHARD DANZIGER, Independent Advisor

4:05 – 4:15

Closing Remarks

SOHINI CHOWDHURY, The Michael J. Fox Foundation

4:30

Cocktail Hour / Poster Viewing

Poster Session

DAY 2

Cognition Endpoints for Parkinson's Disease: A Roadmap for Developing Patient-focused Assessment Tools to Accelerate Drug Development

CHRIS EDGAR, PhD, PCOA

Novel Modeling Approach for Comparing Treatment-naïve and Post-treatment Neuronal Synucleinopathy Progression Using Multi-modal PPMI Data

PIET AARDEN, MSc, Novartis

A Dopamine Clock for Biological Staging of Parkinson's Disease

ROGER GUNN, PhD, XingImaging

Thank You to Our 2026 Sponsors

Gold Sponsor



Silver Sponsors



Bronze Sponsor



Event Partners



If you are interested in sponsoring next year's Parkinson's Disease Therapeutics Conference, contact us at researchpartnerships@michaeljfox.org.



MEIRAGTx

ENGINEERING HOPE

Discover how we're using genetic medicines to help build
a better future for patients with serious diseases.

In collaboration with Hologen AI, MeiraGTX is currently developing AAV-GAD, an investigational genetic medicine to deliver the glutamic acid decarboxylase (GAD) gene to the brain in order to normalize motor circuits and improve symptoms associated with Parkinson's disease without affecting other areas of the brain.



Visit us at
www.meiragtx.com



progress happens together
abcam

Together, we're advancing Parkinson's research

For more than a decade, Abcam's been proud to collaborate with The Michael J. Fox Foundation. Today, as part of Danaher, we're helping connect scientific discovery with the technologies, expertise and partnerships that drive innovation at the speed of life.



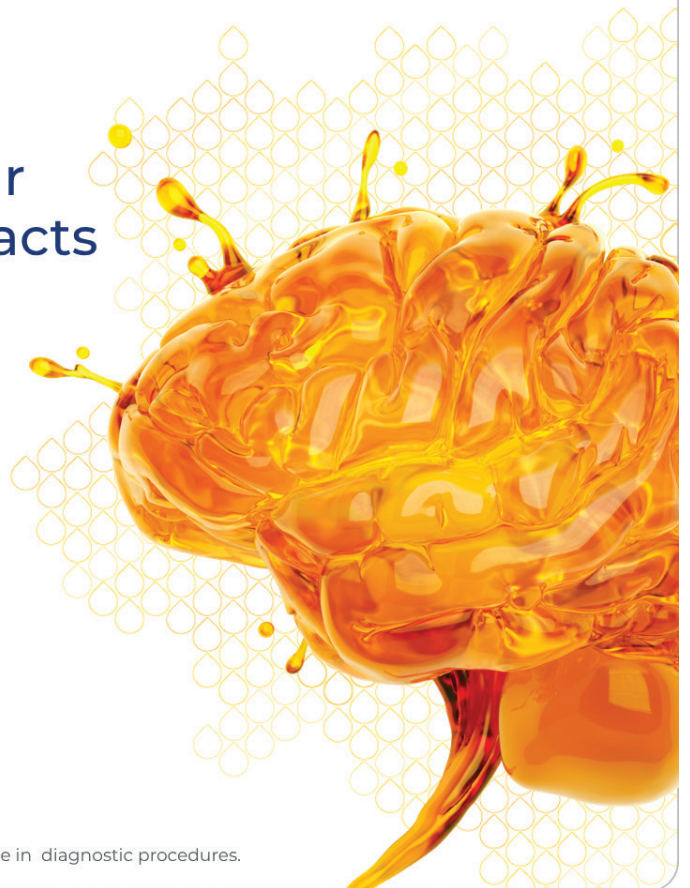
Scan the
QR code to
learn more



Discover the Biology Your Parkinson's Therapy Impacts

Experience NULISA:
High sensitivity & multiplex detection
of key PD pathways including
 α -synuclein, neurodegeneration and
neuroinflammation markers.

Learn more:



For Research Use Only. Not for use in diagnostic procedures.



Developing groundbreaking medicines that transform lives and provide hope to people living with neurodegenerative disorders.

Gain's unique approach enables the discovery of novel, allosteric small molecule modulators that can restore or disrupt protein function. Gain is accelerating drug discovery and unlocking novel disease-modifying treatments for untreatable or difficult-to-treat disorders including neurodegenerative diseases such as GBA1 and idiopathic PD.

PHASE 1B CLINICAL STUDY IN PEOPLE WITH PARKINSON'S DISEASE ONGOING FOR REXACERACT; AN ORALLY-AVAILABLE, SMALL MOLECULE GCase MODULATOR



LEARN MORE ABOUT OUR
ALLOSTERIC SMALL
MOLECULE THERAPIES

Focused on patients. Driven by science.

Breakthroughs only matter when they reach the people they're made for.

Their needs shape our science, our decisions, and our solutions. From global programs to local partnerships, we're breaking down barriers and expanding access to the patients who need it most.

Explore how we're expanding access to lifesaving medicines at [merck.com](https://www.merck.com)





NEUROSYS

INNOVATE FASTER.
DISCOVER BETTER.

PROVIDING PRECLINICAL SERVICES FOR CNS AND PNS DISCOVERY

Our Parkinson's disease platforms combine in vitro and in vivo assays including GBA-focused screening platforms.

CELL-BASED PLATFORMS

- Primary Neurons
- iPSCS
- SH-SY5Y Mutations including GBA mutants

RODENT MODELS

- Age-based
- alpha-synuclein PFF
- GBA impaired

VALIDATED ENDPOINTS:

- α-synuclein Aggregation
- Lysosomal Dysfunction
- Mitochondrial and ER Stress
- Lipid Biomarkers
- Behavior Assessments
- Spatial Transcriptomics
- Immunophenotyping



VALIDATED



TRANSLATIONAL



EFFICIENT



**CONTACT US TO
ACCELERATE YOUR
DRUG DISCOVERY PIPELINE**



neuro-sys.com



info@neuro-sys.com

Xing Imaging

A MITRO COMPANY

MOLECULAR IMAGING CRO | NEURODEGENERATION

**SEE MORE.
MEASURE BETTER.
ADVANCE PD THERAPEUTICS.**

End-to-end molecular imaging support for Parkinson's drug development - from radioligand development and manufacturing to quantitative imaging and multi-site trial execution.

RADIOLIGANDS

Development + cGMP
manufacturing

CLINICAL IMAGING

PET / SPECT + quantitative
analysis

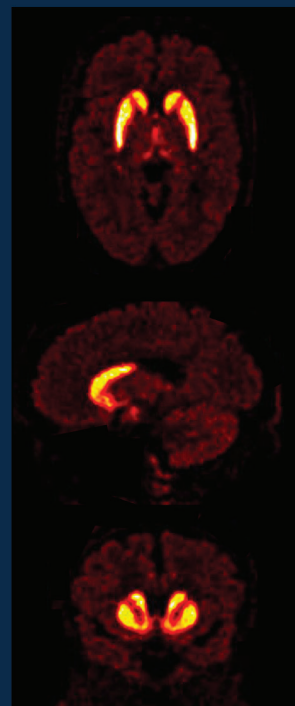
CORE LAB

Phase 1-4 + multi-site imaging
support

LET'S IMAGE WHAT MATTERS.

xingimaging.com | info@xingimaging.com | New Haven, CT

Advanced PET/SPECT imaging | Translational research | Imaging core lab services



2026 PD THERAPEUTICS CONFERENCE

October 14-15 | New York City

Day 1

Wednesday, October 14, 2026

MJFF Keynote Presentation

Mark Frasier, PhD

Chief Scientist

The Michael J. Fox Foundation

Mark Frasier, PhD, joined The Michael J. Fox Foundation in 2006. As chief scientist, Dr. Frasier works with a team of research professionals who stay closely linked to the Parkinson's research community to develop an aggressive and innovative agenda for accelerating research and drug development for Parkinson's disease. This ensures that MJFF research priorities reflect and best serve the ultimate needs of patients. Dr. Frasier regularly meets with academic and industry researchers around the world to identify promising proposals to support, providing troubleshooting and ongoing management of projects as they go forward. He also supports the Foundation's priority interest in developing biomarkers for Parkinson's disease that will accelerate clinical trials of new drugs.

Dr. Frasier earned an undergraduate degree in biochemistry from the University of Dayton and a PhD in pharmacology from Loyola University Chicago. He completed his postdoctoral work in the Neuroscience Discovery Research Group at Eli Lilly, Inc., in Indianapolis, Indiana, where he worked on drug-discovery research in Parkinson's and Alzheimer's disease.

Day 1

Room A: Advancing Targets to Therapies

Gaia Skibinski, PhD

Vice President, Head of Translation Therapeutics
The Michael J. Fox Foundation

Gaia Skibinski, PhD, is vice president and head of translational therapeutics at The Michael J. Fox Foundation for Parkinson's Research. She helps shape and oversee its translational therapeutics strategy, including the Therapeutics program and Targets to Therapies initiative, which accelerate Parkinson's therapies and advance emerging targets. She partners with academia, industry and investors to reduce barriers to drug development and catalyze investment.

Before joining MJFF, she was senior vice president of Biology at Herophilus, where she helped build its human cerebral organoid platform through acquisition by Roche and Genentech and led discovery across neurological and psychiatric diseases. At the Gladstone Institutes, she established human cellular models of LRRK2 and alpha-synuclein, creating a Parkinson's disease drug discovery platform that enabled high-impact collaborations with Amgen, Biogen, Merck, Google Accelerated Science and Verily.

She earned her BA at Oxford and PhD at UCL's Institute of Neurology, where she identified CHMP2B as a cause of frontotemporal dementia.

Allie Vaiana, PhD

Senior Scientific Portfolio Manager
The Michael J. Fox Foundation

Allie Vaiana, PhD, is a neuroscientist and Senior Scientific Portfolio Manager at The Michael J. Fox Foundation (MJFF), where she leads and supports research programs focused on advancing promising biology toward new therapeutic strategies for Parkinson's disease. She helps drive MJFF's Targets to Therapies initiative, working with academic and industry experts to identify high-priority therapeutic targets, define critical evidence gaps and develop strategies to accelerate translation toward drug development. She also works across collaborative research initiatives to advance understanding of Parkinson's biology, including the Collaborative Research Network within Aligning Science Across Parkinson's (ASAP). Allie earned her PhD in neuroscience from UT Health San Antonio, where she investigated mechanisms underlying cancer therapy-induced cognitive decline and potential therapeutic interventions. Before that, she earned her BS in biomedical sciences, with a minor in psychology, from Texas A&M University. Based in Texas, Allie enjoys spending time outdoors with her husband and their two sweet dogs.

Day 1

Room A: Advancing Targets to Therapies

Fiona Ducotterd, PhD

Chief Scientific Officer

Alzheimer's Research UK UCL Drug Discovery Institute

Fiona Ducotterd, PhD, is Chief Scientific Officer at the Alzheimer's Research UK UCL Drug Discovery Institute (UDDI). She is a drug discovery executive with more than 20 years of global experience (including in the UK, US, Japan, China) spanning therapeutics discovery and development, business development and strategic alliances in industry (including MSD, GSK, Eisai, Vertex) and academia (UCL). The UDDI's multidisciplinary team of scientists is discovering new medicines for neurodegenerative diseases in a biotech-like setting, in partnership with leading academic innovators, industry alliances and spinout companies. Dr. Ducotterd holds a BSc in molecular biology from the University of Edinburgh and a PhD in neuroscience from the University of Newcastle. Outside of work, she is mum to 5-year-old daughter Isla and "fur baby" Yuna.

Jonathan Behr, PhD

Independent Advisor

Jon Behr, PhD, is an advisor to The Michael J. Fox Foundation's Targets to Therapies (T2T) initiative, supporting work on neuroinflammatory targets including NOD2, and serves as an independent director of Parkinson's Research Ventures Ltd., a subsidiary of Parkinson's UK. He brings more than 20 years of venture and venture creation experience, including seven years as a Partner at SV Health Investors, where he co-lead the Dementia Discovery Fund (DDF), managed investments and served as a director or observer for multiple biotechnology companies. Previously, he was the first Managing Director of the T1D Fund (a Breakthrough T1D Venture) and held leadership roles at Mass General Brigham Innovation, PureTech Ventures, and Enlight Biosciences, where he co-founded and supported seven companies. Dr. Behr earned a PhD in biological engineering from MIT as a Howard Hughes Medical Institute fellow and a BS in bioengineering, summa cum laude, from Rice University.

Day 1

Room B: PPMI and Enabling Clinical Trials in PD

Catherine Kopil, PhD

Senior Vice President and Head of Clinical Research
The Michael J. Fox Foundation

Catherine (Katie) Kopil, PhD, is the senior vice president and head of clinical research at The Michael J. Fox Foundation where she focuses on building the Foundation's capacity as an unprecedented stakeholder in Parkinson's drug development: a nimble, patient-focused problem-solver whose efforts are demonstrably accelerating progress toward treatment breakthroughs. She leads a team investing in solutions to de-risk clinical development for Parkinson's and related disorders. Dr. Kopil and her team support field-enabling efforts including seminal natural history studies like the Parkinson's Precision Medicine Initiative (PPMI), alignment on regulator-acceptable endpoints for clinical trials and integration of patient perspectives throughout R&D. Dr. Kopil earned a BA in psychology from Princeton University, then completed doctoral and postdoctoral training in neuroscience and bioengineering, respectively, at the University of Pennsylvania. Her research focused on brain injury that occurs during acute trauma such as cardiac arrest and concussion. She also worked to speed clinical research as a clinical trial coordinator at Memorial Sloan Kettering Cancer Center in New York City, where her dual passions for science and serving patients first intersected.

Day 1

Room B: PPMI and Enabling Clinical Trials in PD

Tanya Simuni, MD, FAAN

Arthur C. Nielsen Professor of Neurology
Division Head, Parkinson's disease and Movement Disorders Center
Northwestern University Feinberg School of Medicine

Tanya Simuni, MD, leads a comprehensive movement disorders center at Northwestern University Feinberg School of Medicine, recognized as a Center of Excellence by the Parkinson's Foundation, the Huntington's Disease Society of America and the Wilson Disease Association, and as a training model in the region.

She is an internationally recognized expert in the design and implementation of Parkinson's disease (PD) clinical trials focused on disease modification, and she is the lead author of the new biological definition and staging framework for neuronal synuclein disease (NSD). She serves on the leadership team of The Michael J. Fox Foundation-sponsored Parkinson's Precision Medicine Initiative (PPMI), the largest PD biomarker initiative, where she also serves as the principal investigator for the first platform trial to test therapeutics in a biologically defined prodromal population (P2P). She serves on steering committees for several international PD clinical trials, as well as on several committees of the Parkinson Study Group and the Parkinson's Foundation. Dr. Simuni is the site principal investigator for the Network for Excellence in Neuroscience Clinical Trials (NEXT). She has authored more than 150 publications in peer-reviewed scientific journals and has lectured nationally and internationally on PD and other movement disorders.

In addition to her research career, Dr. Simuni is deeply committed to educating the next generation of physicians and to patient advocacy. She is an active member of the American Academy of Neurology, the American Neurological Association, the International Parkinson and Movement Disorder Society and the Parkinson Study Group.

Day 1

Room B: PPMI and Enabling Clinical Trials in PD

Kenneth Marek, MD

President and Senior Scientist

The Institute for Neurodegenerative Disorders

Kenneth Marek, MD, is president and senior scientist at the Institute for Neurodegenerative Disorders. Dr. Marek's major research interests include identifying biomarkers for early detection, assessing disease progression and developing new treatments for neurodegenerative disorders including neuronal synuclein disease (Parkinson's disease and dementia with Lewy bodies), Alzheimer's disease and related neurodegenerative disorders. His specific interest has been in vivo neuroreceptor imaging biomarkers. He has authored numerous neurology and neuroscience publications on these topics. Dr. Marek has been, and continues to be, the principal investigator of several ongoing multi center international studies, including the Parkinson's Precision Medicine Initiative (PPMI) and the Parkinson Associated Risk Syndrome (PARS) study.

Dr. Marek serves as a special scientific advisor to The Michael J. Fox Foundation. He has held leadership roles in several organizations focused on neurodegenerative disorders and has received numerous grants to support his work in Parkinson's, Alzheimer's and Huntington's disease. Among his honors is the Robert A. Pritzker Prize for Leadership in Parkinson's Research. He also co-founded Molecular NeuroImaging, LLC, and Xing imaging LLC, companies providing discovery and clinical neuroimaging research services.

Day 1

Room C: MJFF Biomarker Toolkits and Resources

Christine Swanson-Fischer, PhD

Director of Translational Biomarkers

The Michael J. Fox Foundation

Christine Swanson-Fischer, PhD, is director of translational biomarkers at The Michael J. Fox Foundation. She and her team drive translational biomarker strategy including accelerating biomarkers as fit-for-purpose tools to guide therapeutic development. She routinely partners with academic, industry, investors, federal and nongovernmental organizations to advance strategic biomarker and precision medicine initiatives.

Prior to joining MJFF, she was a scientist and program director at the National Institutes of Health where she led biomarker and therapeutic development initiatives in Parkinson's disease and related disorders.

She earned her PhD at the University of Wisconsin Madison in neuroscience and served as a postdoctoral fellow at the University of Pennsylvania, where she employed targeted and unbiased proteomic profiling to enable biomarker discovery and validation in Parkinson's disease.

Ariana Farrand, PhD

Senior Scientific Portfolio Manager

The Michael J. Fox Foundation

Ariana Farrand, PhD, is a scientist on the Translational Biomarkers team at The Michael J. Fox Foundation, where she manages projects and develops strategy around advancing biofluid biomarkers related to immune and mitochondrial dysfunction in Parkinson's disease. Since joining MJFF in 2022, she has managed research portfolios spanning preclinical therapeutic and biomarker development across several pathway areas. Her recent work includes supporting biomarker strategy across therapeutic programs, developing biomarker recommendations for priority therapeutic targets and working to advance understanding of immune changes in Parkinson's disease. Dr. Farrand's research background includes neuroinflammation, oxidative stress, neuroprotection and brain stimulation. She earned her PhD in neuroscience from the Medical University of South Carolina, where she studied the potential use of vagus nerve stimulation for Parkinson's. She then completed a postdoctoral fellowship at East Tennessee State University, focused on understanding mechanisms of action for vagus nerve stimulation, before joining MJFF.

Day 1

Room C: MJFF Biomarker Toolkits and Resources

Katrina Paumier, PhD

Senior Director of Medical Affairs (Parkinson's)

Octave Bioscience

Katrina Paumier, PhD, is a neuroscientist with more than 20 years of experience advancing neurodegenerative therapeutics from translational research through clinical development. She is senior director of medical affairs (Parkinson's) at Octave Bioscience, where she leads the strategy and validation of a custom multi-analyte proteomics panel and serves as the principal investigator for a \$10 million grant from The Michael J. Fox Foundation (MJFF).

Her background spans industry and academic leadership, including positions at Spur Therapeutics, Sage Therapeutics and Washington University, as well as a postdoctoral fellowship at Pfizer. She has made significant scientific contributions to Parkinson's disease research, notably in alpha-synuclein pathology and biomarker development, resulting in 32 peer-reviewed publications and more than \$6 million in secured research funding. She also serves as a scientific advisor and grant reviewer for MJFF.

Day 1

Room D: Multimodal Data and Cohorts to Enable Precision Medicine

Samantha Hutten, PhD

Principal Biomarker Scientist

The Michael J. Fox Foundation

Samantha Hutten, PhD, has spent the last 14 years leading biomarker strategy at The Michael J. Fox Foundation. In her current role, she develops and leads programs focused on biomarker discovery across omics, imaging and advanced analytics to support disease understanding, advance the pipeline and address critical field-wide challenges and gaps. Dr. Hutten collaborates closely with partners across Parkinson's Precision Medicine Initiative (PPMI) and Global Parkinson's Genetic Program (GP2), to ensure synergy across programs. She also serves as co-chair of the Accelerating Medicines Partnership Parkinson's Disease and related Disorders (AMP-PDRD) Biologics Working Group.

After graduating with honors from Vassar College, Dr. Hutten completed her PhD at Albert Einstein College of Medicine, studying the role of dysfunctional autophagy in Parkinson's Disease. Her work identified the mechanism by which LRRK2 impairs cellular clearance via chaperone-mediated autophagy. Dr. Sutton is passionate about fueling the biomarker pipeline to support clinical trials.

Kendall Van Keuren-Jensen, PhD

Vice President for Research and Professor

Translational Genomics Research Institute

Kendall Van Keuren-Jensen, PhD, is vice president for research and professor of bioinnovation at the Translational Genomics Research Institute (TGen). Her research focuses on developing molecular approaches to understand and monitor human disease, with particular emphasis on neurodegeneration. Her laboratory develops technologies for analyzing RNA, extracellular vesicles and other molecular signals in tissues and biofluids, with the goal of connecting genetic risk and cellular dysfunction to biomarkers measurable in living individuals. Dr. Van Keuren-Jensen leads and collaborates on large multidisciplinary studies of Parkinson's disease, Alzheimer's disease and related neurodegenerative disorders, including efforts within the Global Parkinson's Genetics Program (GP2) and the Aligning Science Across Parkinson's (ASAP) initiative. Her current work integrates genetics, transcriptomics, proteomics, single-cell approaches and extracellular vesicle biology to investigate disease mechanisms, define biologically meaningful patient subtypes and develop biomarkers that can support therapeutic development and precision medicine.

Day 1

Room D: Multimodal Data and Cohorts to Enable Precision Medicine

Chris Whelan, PhD

Adjunct Faculty, Founding Principal

Royal College of Surgeons in Ireland, Ignition Scientific

Christopher (Chris) D. Whelan, PhD, is a neuroscience and multi-omics researcher based in Boston, Massachusetts. From 2016 to 2025, he led scientific teams at Pfizer, Biogen and Johnson & Johnson (J&J), most recently as director of molecular neuroscience data science at J&J. While at Biogen, he founded and chaired the UK Biobank Pharma Proteomics Project (UKB-PPP), a consortium of 13 pharmaceutical companies that generated one of the largest population-scale proteomics datasets to date. At J&J he grew UKB-PPP to 14 companies and raised over \$50M from pharma and academic sources. He then left industry to divide his time between independent consulting, through Ignition Scientific, and academia, as Honorary Faculty at the Royal College of Surgeons in Ireland (RCSI). Chris received his PhD in diagnostics and therapeutics for human disease, specializing in neuroscience and human genetics, from RCSI. He then completed a postdoctoral fellowship at the University of Southern California, where he formed and led the ENIGMA-Epilepsy neuroimaging consortium.

Day 1

Room E: Patient-Centered Strategies to Enable Therapeutic Development

Maggie Kuhl

Vice President and Head of Patient Engagement

The Michael J. Fox Foundation

As vice president and head of patient engagement at The Michael J. Fox Foundation, Maggie Kuhl leads a team that engages community partners and gathers patient experience data to support patient-focused drug development. Her team also pilots and refines recruitment and retention methods to enable faster trials. Ms. Kuhl directs recruitment strategy for the Foundation's Parkinson's Precision Medicine Initiative, a longitudinal observational study with remote screening and enrollment for thousands of participants at 50 international sites. Before joining the Foundation in 2013, she worked in communications at the National Institutes of Health.

Day 1

Accelerating Parkinson's Disease Through Shared Resources

Nicole Polinski, PhD

Director of Research Resources
The Michael J. Fox Foundation

Nicole K. Polinski, PhD joined The Michael J. Fox Foundation for Parkinson's Research in 2016 and serves as Director of Research Resources on the Translational Research team. In this role, Nicole has a broad perspective on the research resources, capabilities, and programs available across MJFF. She specifically leads the MJFF Research Tools Program which seeks to improve research rigor and reproducibility by developing and distributing preclinical tools and models that can be accessed by the community and used in Parkinson's disease research and therapeutic development. Nicole is also a subject-matter expert and key opinion leader in preclinical Parkinson's disease research tools and models, advising on model/tool selection, validation, fit-for-purpose use, and the development of new resources to address critical gaps in the field.

National Parkinson's Project: A Federal Framework to Accelerate Progress Against Parkinson's Disease and Related Disorders

Danielle Mutone-Smith, PhD

Director of Federal Government Relations
The Michael J. Fox Foundation

Danielle Mutone-Smith, PhD is Director of Federal Government Relations at The Michael J. Fox Foundation for Parkinson's Research, where she leads federal advocacy efforts to advance the Foundation's public policy priorities. In this role, she works across Congress and the Executive Branch to increase federal investment in Parkinson's research, support implementation of the National Parkinson's Project, and advance policies that improve access to care and treatments for people living with Parkinson's disease.

Prior to joining the Foundation, Danielle held roles in government, public affairs, and policy, providing firsthand experience working within the federal policy environment and engaging a broad range of stakeholders. She frequently works with congressional offices, federal agencies, researchers, and patient advocates to advance shared priorities and foster collaboration across sectors. Danielle brings a practical understanding of the federal policymaking process and the opportunities it presents to accelerate research and improve outcomes for people living with Parkinson's.

Day 1

Advancing GBA1 Biomarkers: The Industry GBA1 Biomarker Consortium

Fernanda Cerqueira, PhD

Senior Scientific Portfolio Manager

The Michael J. Fox Foundation

Fernanda Cerqueira is a biochemist and biomarker strategist at the Michael J. Fox Foundation, where she leads the endolysosomal biomarker portfolio. She holds a PhD in Biochemistry and brings expertise in aging and metabolism to assay development and biofluid biomarker validation for clinical development in LRRK2 and GBA1 programs. She also builds biomarker toolkits to support companies developing therapies that target multiple components of the endolysosomal system. Drawing on five years of biotechnology experience, Fernanda collaborates across MJFF's Cohort, Alliances, Discovery, Imaging, and Therapeutics teams, translating biomarker data into actionable insights for high-priority programs.

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

SESSION CHAIR

Magdalene Moran, PhD

Chief Executive Officer

Topo Therapeutics

Magdalene Moran, PhD, is chief executive officer of Topo Therapeutics. She received her PhD in neurobiology from Harvard University, where she was advised by ion channel expert David Clapham, PhD. For more than 20 years, Dr. Moran has led drug discovery efforts on transmembrane protein targets implicated in pain, pulmonary, central nervous system (CNS) and renal diseases, advancing four programs from inception into the clinic with large pharma partners. Most recently, she was the president and chief scientific officer of Caraway Therapeutics, where she built out operations, drove scientific strategy and led drug discovery efforts for multiple lysosomal ion channel targets in the Parkinson's disease, from the company's founding through its acquisition by Merck in November 2023.

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

Brain Shuttle-Enabled Delivery of a TrkB Agonist Antibody for Disease Modification in Parkinson's Disease

Pawel Stocki, PhD

Vice President of Research

Ossianix

Pawel Stocki, PhD, is vice president of research at Ossianix, where he leads the development of brain-targeted biologics enabled by the company's proprietary TXP1 brain shuttle platform. His research focuses on overcoming the blood-brain barrier (BBB) to enable systemic delivery of therapeutics for neurodegenerative diseases, including antibody-based approaches targeting clinically validated central nervous system (CNS) pathways. Before joining Ossianix in 2015, he worked on intracellular delivery technologies at MedImmune and served as research director at Psynova Neurotech. He is an inventor on multiple patents covering BBB transport technologies and CNS-targeted therapeutics.

Abstract

Neurotrophic support through BDNF-TrkB signalling has long been considered a promising therapeutic strategy for Parkinson's disease (PD), but clinical translation has been limited by poor blood-brain barrier (BBB) penetration. We developed a brain-penetrant TrkB agonist antibody by combining a humanized TrkB agonist monoclonal antibody with a proprietary brain shuttle technology that enables efficient central nervous system (CNS) delivery.

The brain shuttle-enabled antibody retained potent agonist activity and substantially increased CNS exposure, resulting in robust TrkB pathway activation in vivo, including ERK signalling in the substantia nigra. In the 6-OHDA model, treatment preserved TH+ dopaminergic neurons. In the α -synuclein PFF/CBE model, weekly dosing improved motor performance, prevented α -synuclein accumulation through enhanced microglial phagocytic activity and provided full protection of nigral dopaminergic neurons. Preliminary non-human primate studies demonstrated a favourable pharmacokinetic and safety profile.

These findings demonstrate that systemic TrkB activation can modify key pathological and functional features of PD when sufficient brain exposure is achieved. The data support further development of this brain-penetrant TrkB agonist antibody as a potential disease-modifying therapy for PD.

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

20S Proteasome Activation Program

Diogo Feleciano, PhD

Co-Founder and Chief Scientific Officer

Booster Therapeutics

Diogo R. Feleciano, PhD, is co-founder and chief scientific officer of Booster Therapeutics, a preclinical-stage biotech company developing oral, brain-penetrant small molecules that restore proteasome function to treat Parkinson's Disease. An entrepreneur passionate about translating science into transformative treatments, he has led the company since its founding in 2020. In 2024, Booster raised a \$15M from Apollo Health Ventures and Novo Holdings.

Dr. Feleciano completed his undergraduate degree in biochemistry at the University of Coimbra in Portugal in 2013, then moved to the UK to work in ocular research at Imperial College London. In 2015, he moved to Berlin, Germany, to pursue his PhD in biochemistry from the Freie Universität Berlin, focusing on the impact of proteasome function during aging and neurodegenerative diseases. In 2019, he became an entrepreneur in residence at Apollo Health Ventures, which led to the founding of Booster.

Abstract

Targeting proteasomal degradation leads to increased clearance of misfolded proteins, protection of nigrostriatal dopaminergic neurons and ultimately slows down or prevents Parkinson's disease (PD) progression. Booster's approach targets this well-known biological pathway using an innovative approach with the goal of identifying a small molecule proteasome activator to be developed as a disease-modifying therapy for PD. The project will focus on 1) defining a translational strategy to identify biomarkers of target engagement and efficacy early in the drug discovery, and 2) identifying and characterizing a novel, small molecule candidate ready to enter investigational new drug (IND)-enabling studies.

Development of Small-Molecule Inhibitors of the Mitochondrial Permeability Transition Pore for the Treatment of Parkinson's Disease and ALS

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

Development of Small-Molecule Inhibitors of the Mitochondrial Permeability Transition Pore for the Treatment of Parkinson's Disease and ALS

Richard Rutter, PhD

Chief Scientific Officer

NRG Therapeutics

Richard Rutter, PhD, is an expert drug discovery pharmacologist with 20 years of experience in the pharmaceutical and biotechnology industries. He began his career at the Neuroscience Research Centre at Merck Sharp & Dohme and later joined the Neurodegeneration Research Centre at GlaxoSmithKline in Singapore, where he was director of biology. He has led teams that delivered data packages supporting the progression of multiple compounds into clinical development for neurodegenerative diseases. His expertise spans the early stages of drug discovery, including target identification, lead optimization and the advancement of compounds into Phase I clinical studies. More recently, Dr. Rutter moved into biotechnology, spending two years as chief scientific officer (CSO) at Auspherix, developing novel antibiotics before co-founding NRG Therapeutics in 2018, where he serves as CSO.

Abstract

NRG Therapeutics is developing a small-molecule inhibitor of the mitochondrial permeability transition pore (mPTP) as a potential disease-modifying treatment for Parkinson's disease. The approach protects mitochondria from the toxic effects of oligomeric alpha-synuclein together with mitochondria Ca²⁺-overload in dopaminergic neurons. The inhibitor acts through NLRX1, a novel protein modulator of the mPTP and an innate immune receptor. The lead inhibitor, NRG5051, has demonstrated beneficial effects in the AAV-A53T α-synuclein rat model and in Pink1/Parkin double-knockout rats. Neuroprotective effects have also been demonstrated in preclinical models of acute CNS injury and ALS, with profound effects on innate immune signaling in NLS-TDP-43 transgenic mice. Phase I studies assessing safety, tolerability and target engagement in healthy volunteers are ongoing. Additional enabling studies aim to identify and validate biomarkers of target engagement and mitochondrial function for incorporation into future clinical trials in people with Parkinson's disease.

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

Development of a TRPML1 Agonist for GD-PD and Beyond

Leon Murphy, PhD

Chief Scientific Officer

Casma Therapeutics

Leon Murphy, PhD, is an industry-leading expert in autophagy, mTOR and PI3-kinases and has broad expertise building and leading teams that develop novel therapeutics for cancer, neurodegeneration and metabolic disorders. At Novartis he was a member of teams that supported the development of Piqray® for advanced breast cancer and Afinitor® for epilepsy in people with tuberous sclerosis complex. He is currently the chief scientific officer at Casma Therapeutics, a Massachusetts-based biotechnology company focused on the autophagy-lysosomal system. Before joining Casma, Dr. Murphy was an entrepreneur-in-residence at Third Rock Ventures where he helped develop the scientific and business strategy for Casma Therapeutics. He is recognized as a leader in the autophagy-lysosomal field and has published multiple papers in high-impact journals. His formal training was in biochemistry and cell biology.

Abstract

There are approximately 300 Type 1 Gaucher's patients with Parkinson's disease (GD-PD) in the US. GD-PD patients are typically excluded from PD clinical trials, and their neurological symptoms do not benefit from Cerezyme due to the lack of brain-penetration. In addition to GD-PD, there are upwards of 150,000 GBA1-associated PD (GBA-PD) patients in the US. Only symptomatic treatment options are available for GD-PD and GBA-PD patients. Both diseases share a common molecular deficit in GCase, which leads to a primary lysosomal storage defect, neuronal pathology and overall progression. Brain-penetrant small molecule agonists for the lysosomal TRPML1 channel have compelling efficacy in preclinical models of GD-PD and GBA-PD and have potential to be disease-modifying in patients. Since lysosomes have aberrant pH and an accumulation of toxic lipids in GD-PD and GBA-PD, pharmacological enhancement of TRPML1 is an attractive approach to normalize endolysosomal function and restore neuronal homeostasis in patients. This presentation will summarize the preclinical evidence to support development of CSM-101 and outline the clinical strategy to reach human validation.

Day 2, Session 1

Emerging Therapeutic Strategies in Parkinson's Disease

HER-096: A Subcutaneous CDNF-Derived Peptide for Parkinson's Disease — Rationale and Phase Ib Results

Henri Huttunen, PhD

Chief Scientific Officer and Co-Founder
Herantis Pharma Plc

Henri J. Huttunen, PhD, is chief scientific officer and co-founder of Herantis Pharma Plc, a clinical-stage biotechnology company developing therapies for neurodegenerative diseases. He leads the development of HER-096, a subcutaneously administered, brain-penetrant peptide derived from cerebral dopamine neurotrophic factor (CDNF), which is advancing toward Phase 2 clinical trials in early Parkinson's disease. His work integrates mechanism-of-action research, translational biomarkers and digital measures of motor progression.

Dr. Huttunen has more than two decades of experience in neurodegeneration research and therapeutic translation. He received his PhD in biochemistry from the University of Helsinki and held research positions at Orion Pharma, Massachusetts General Hospital, and Harvard Medical School before leading a research group at the Neuroscience Center, University of Helsinki.

Abstract

Cerebral dopamine neurotrophic factor (CDNF) modulates the unfolded protein response (UPR) through its central regulator GRP78, potentially restoring coordinated adaptive stress responses rather than targeting individual UPR signaling branches. Intracranial administration of recombinant CDNF has previously been evaluated in patients with Parkinson's disease (PD), providing clinical experience with this therapeutic approach and exploratory signals consistent with neuroprotective or neurorestorative activity.

HER-096 is a ~1 kDa synthetic peptidomimetic derived from the UPR-modulating region of CDNF, designed to reproduce its protective activity without intracranial administration. Given subcutaneously with intermittent dosing, HER-096 preserves dopaminergic neurons, normalizes striatal dopamine and improves motor function in PD models.

Two Phase I studies are complete: single ascending doses in 68 healthy volunteers and four weeks of repeated dosing in 24 people with PD. HER-096 was safe and well tolerated, with predictable plasma pharmacokinetics and demonstrated CSF exposure. Multi-matrix biomarker profiling showed dose-related biological responses consistent with the proposed mechanism of action, while direct evidence of CNS target engagement remains to be established.

This presentation will outline how the totality of that evidence supports testing disease modification in patients, and how the remaining uncertainties are addressed in a Phase II study planned to start in the first half of 2027.

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

SESSION CHAIR

Johannes Streffer, MD

Head of Global Clinical Development (SVP)

Lundbeck

Johannes Streffer, MD, is senior vice president and head of global clinical development at Lundbeck and a visiting professor in the Department of Biomedical Sciences at the University of Antwerp. A board-certified neurologist and psychiatrist, he has more than 30 years of experience in clinical research and drug development across neuropsychiatric and neurodegenerative disorders.

Before joining Lundbeck, Dr. Streffer served as chief medical officer at AC Immune and as vice president and head of translational medicine neuroscience at UCB, and he held multiple leadership roles in neuroscience development at Janssen R&D. His work has focused on translational neuroscience, biomarkers and the development of innovative therapies for neurodegenerative diseases, including Alzheimer's disease and Parkinson's disease.

Dr. Streffer is a member of the World Dementia Council and has led major international research collaborations in neurodegeneration. He has authored more than 100 peer-reviewed publications in leading scientific journals, including *Brain*, *Neuron*, *The Lancet* and *Nature Medicine*.

Jamie Eberling, PhD

Senior Director of Imaging Research

The Michael J. Fox Foundation

Jamie Eberling, PhD, is the senior director of imaging research at The Michael J. Fox Foundation (MJFF) and oversees the Foundation's imaging portfolio with a particular emphasis on PET tracer development. She is responsible for building and advancing the alpha-synuclein tracer development program, one of the highest research priorities for MJFF.

Before joining MJFF, Dr. Eberling was a research scientist at the Lawrence Berkeley National Laboratory where she used PET imaging to evaluate efficacy of gene therapy approaches for Parkinson's disease. Dr. Eberling received her BS and PhD in biological physiology from the University of California, Berkeley.

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

Detect PD: Simple Blood-Based Testing for Development and Progression of Neuronal Alpha-Synuclein Disease

Julia Carrasco Zanini Sanchez, PhD

Lecturer

Queen Mary University of London

Julia Carrasco Zanini Sanchez, PhD, is a lecturer in multiomic science at the Precision Healthcare University Research Institute (PHURI), Queen Mary University of London. Dr. Carrasco Zanini Sanchez's research focuses on how integration of multiomics with electronic health records can be applied to improve targeted screening, prediction and prognostic strategies for a diverse range of common and rare diseases. She received her PhD from the University of Cambridge, as part of the Wellcome Trust MRes + PhD program in metabolic and cardiovascular disease. Her work on broad-capture proteomics spanning common and rare disease etiology and biomarker identification has led to several industry and academic collaborations and was recognized with the UK Biobank Early Career Researcher of the Year award in 2023.

Celine Roeder

MD/PhD Student

Berlin Institute of Health at Charité

Celine Roeder is a final-year medical student at Charité Berlin and a doctoral candidate in Claudia Langenberg's Computational Medicine group at the Berlin Institute of Health at Charité. Since November 2025, she has also been an Honorary Research Fellow at the Precision Healthcare University Research Institute (PHURI), Queen Mary University of London. Her research uses proteogenomic approaches to uncover the molecular architecture of neurodegenerative diseases, with a focus on proteomic biomarkers in Parkinson's disease using data from the Parkinson's Precision Medicine Initiative (PPMI). She previously conducted research at the Institute of AI for Health, Helmholtz Munich and gained industry experience in several healthtech start-ups. Celine studied medicine at the Technical University of Munich and Charité Berlin, she also holds an honors degree in technology management from the Center for Digital Technology and Management (CDTM).

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

Abstract

Background. Proteomic profiling of cerebrospinal fluid (CSF) and plasma holds promise for biomarker discovery in Parkinson's disease (PD). However, protein measurements are shaped not only by disease but also by a wide range of demographic, clinical, environmental, genetic and technical factors. Understanding these sources of variation and how they differ across biological matrices is essential to identify robust, disease-relevant biomarkers more suitable for translational efforts.

METHODS

We used baseline data from the Parkinson's Precision Medicine Initiative (PPMI), including healthy controls, prodromal individuals and people with sporadic and genetic forms of PD. CSF and plasma proteins were measured using two affinity-based platforms, Alamar NULISeq (n = 360 proteins) and Olink (n = 5460) in up to 1,898 individuals.

We combined three complementary approaches, applied separately to each platform and matrix: (1) association testing of protein levels with PD-related outcomes, including disease stage, genetic subtype and polygenic risk; (2) machine-learning modelling to derive sparse signatures for early detection or prognosis of PD and its subtypes; and (3) systematic quantification of the contribution of clinical, genetic, imaging, demographic and technical influences on each protein's CSF or plasma variation.

RESULTS

We identified between 4-220 and 3-431 proteins associated with PD-related outcomes in CSF and plasma across both platforms. For example, up to 98 proteins were associated with LRRK2+ carrier status in CSF. In contrast, machine learning modeling enabled prioritization of a 10-protein CSF signature that was able to discriminate LRRK2 carrier status against sporadic PD cases (AUROC = 0.79). Systematic characterization of biological and technical influences revealed widespread influences of demographic factors such as age and sex across the proteome, whereas PD-relevant factors, such as genetic status, dopaminergic imaging and medication, explained a meaningful share of variance for a smaller subset of proteins, with matrix-specific differences. Detailed characterization of biological and technical influences enabled prioritization of protein biomarkers through identification of likely underlying mechanisms, confounding factors, stability and technical robustness.

CONCLUSIONS

Data driven approaches in deeply characterized disease cohorts can accelerate candidate biomarkers prioritization for PD and informs the design and interpretation of future multi-platform proteomic studies.

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

Polygenic Risk Scores in Neuronal Alpha-Synuclein Disease Stage Progression

Manuela Tan, PhD

Postdoctoral Research Fellow

University College London

Manuela Tan, PhD, is a postdoctoral research fellow at Oslo University Hospital and Queen Mary University of London. She works within the Global Parkinson's Genetics Program (GP2), where she is one of the junior co-leads of the Prodromal Working Group and the Cohort Integration Working Group. Her research focuses on Parkinson's disease genetics, and she has been involved in leading large-scale genome-wide association studies of Parkinson's disease progression. Before her current position, she completed her PhD at University College London with Huw Morris, PhD, and now works with Alastair Noyce, PhD, to coordinate and lead research in the prodromal group of GP2.

Abstract

Neuronal alpha-synuclein disease (NSD) is a new framework for thinking about Parkinson's disease (PD), using a disease-relevant biomarker neuronal alpha-synuclein, confirmed with the alpha-synuclein Seed Amplification Assay (SAA). In recent years, a staging system has been proposed for NSD, called the NSD Integrated Staging System (NSD-ISS). In this, individuals progress from Stage 1a (the presence of neuronal alpha-synuclein alone, without any other clinical symptoms), Stage 1b (neuronal alpha-synuclein with dopaminergic dysfunction but no other symptoms), Stage 2a (subtle clinical symptoms but without functional impairment), Stage 2b (subtle clinical symptoms with dopaminergic deficit, but no functional impairment), to Stage 3 and onwards (dopaminergic dysfunction and clinical symptoms with increasing functional impairment). Our goal was to explore the role that common genetic variation related to sporadic PD plays in NSD staging. Previous data suggests that PD polygenic risk scores (PRS) are not associated with SAA status.

In this project, we analyzed the PD PRS against NSD stage progression in the Parkinson's Progression Markers Initiative (PPMI) study. We included 1,421 individuals of European ancestry from PPMI who were NSD positive and had been genotyped on the Neurobooster Array, conducted by Global Parkinson's Genetics Program (GP2). Using time-to-event analysis to next stage increase, we show that PD PRS are associated with NSD stage progression only in individuals at Stage 2 at baseline (N = 665), progressing to higher stages. The PD PRS was not associated with NSD stage progression when considering individuals at all baseline stages (N = 1421). These findings held when analyzing European and Ashkenazi Jewish ancestry individuals separately. This data could suggest that PD genetics influences progression from prodromal/pre-clinical stages to functional impairment in NSD positive individuals. The PRS could also influence future versions of the NSD-ISS.

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

Multivariate Latent Disease Progression Modeling in Neuronal Alpha-Synuclein Disease

Barbara Wendelberger, PhD

Senior Statistical Scientist

Berry Consultants

Barbara Wendelberger, PhD, is a senior statistical scientist at Berry Consultants, where she focuses on innovative statistical approaches to address complex challenges in clinical trial design. In her work, she designs Bayesian adaptive clinical trials for numerous therapeutic areas and specializes in leveraging natural history data on biomarkers and clinical endpoints to model disease progression. She is passionate about translating a deeper biological understanding of a disease to a more efficient and informative clinical trial, particularly in progressive, neurodegenerative and rare diseases. Before joining Berry Consultants in 2016, she earned her PhD and MS from the University of Wisconsin and her BS from the University of Notre Dame.

Abstract

Population heterogeneity is a key challenge for both natural history studies and clinical trials. Recently, a biological definition, and associated staging system, of Neuronal α -Synuclein Disease (NSD) has emerged to address the intrinsic limitations of a clinical diagnosis of Parkinson's disease and reduce some of this heterogeneity. Even with this staging system, there is uncertainty about how patients progress through the underlying disease process.

A cascade of biological and clinical markers captures changes that occur across the spectrum of NSD. Disease modeling can be used to capture this accumulating pathology and quantify the underlying disease state. This enables more targeted study of disease populations at specific points in disease.

Here, we build latent time joint disease progression models (DPMs) for NSD using data from PPMI natural history study participants across the NSD continuum. These models capture the underlying disease process of NSD as a function of clinical and biological outcome measures to quantify a participant's latent "disease age." Quantification of this latent disease age illuminates the timing of pathological measurements in the disease course and captures the expected rate and variability of progression in an outcome measure. It also enables exploration of differential rates of progression in subgroups, while reducing population heterogeneity. These features aid in progressing therapeutic development through clinical trials. DPMs can be used in disease age-based inclusion/exclusion criteria, as a tool for patient-level simulation and clinical trial design, and as an innovative analysis method to improve trial efficiency and power.

Day 2, Session 2

Enabling Precision Medicine Approaches in Clinical Development

In constructing our DPM, we considered PPMI data from the population of NSD positive individuals relative to data contributed from NSD negative healthy controls to understand differences in disease progression. The multivariate DPM characterizes the trajectory of pathological events that result in disease manifestation by jointly estimating multiple biomarker and clinical outcome measures within participants to estimate their latent disease age and simultaneously estimate timing and rates of progression for each outcome across the NSD spectrum.

We have explored disease progression for key subgroups, including PPMI recruitment group, genetic group and sex, by visualizing their outcome measure trajectories as a function of latent disease age.

The DPMs presented here provide a better understanding of the pathological cascade that occurs in NSD. We will demonstrate results highlighting the early pathological behavior captured by DAT imaging and its relation to clinical symptoms. Such results have important implications for clinical trial design. Additionally, DPMs provide powerful tools to improve therapeutic development, and we will highlight how these tools can be used.

Day 2, Session 3

“Hot Topics” — The Future of Durable Therapies in Parkinson’s Disease

SESSION CHAIR/ MODERATOR

Brian Fiske, PhD

Chief Scientist

The Michael J. Fox Foundation

Brian Fiske, PhD, joined The Michael J. Fox Foundation in 2004. As chief scientist, Dr. Fiske works with a team of professionals who stay closely linked to the Parkinson’s community in order to develop an aggressive and innovative strategic vision for accelerating research and drug development for Parkinson’s disease. This ensures that MJFF priorities reflect and best serve the ultimate needs of patients.

Dr. Fiske earned an undergraduate degree in biology from Texas A&M University and a PhD in neuroscience from the University of Virginia. After completing postdoctoral research at Columbia University, he spent several years as an editor for the scientific journal, *Nature Neuroscience*, before coming to the Foundation to help bring new treatments to people with Parkinson’s.

Dr. Fiske is passionate about the mission of the Foundation. In addition to his work as a Foundation scientist, he has been an active Team Fox member, running the New York Marathon and other race event to raise critical funds for research.

PANELISTS

Patrik Brundin, MD, PhD

Therapeutic Area Leader for Movement Disorders

Roche

Patrik Brundin, MD, PhD, is a prominent neuroscientist dedicated to Parkinson’s disease research, inspired early in life by his father’s journey with the condition. With an MD and a PhD from Lund University, he spent decades in academia in Sweden and in the United States pioneering cell transplantation and discovering how alpha-synuclein aggregates spread and contribute to disease progression.

Dr. Brundin also brings vital industry experience, having spent nearly five years leading movement disorders research and early drug development at Roche, where therapeutic candidates designed to slow progressive worsening of symptoms are in advanced clinical trials. He has co-founded biotech startups, consulted for major pharmaceutical companies and led numerous international research networks.

A global authority with over 450 scientific publications (cited 75,000 times), Dr. Brundin served as inaugural co-editor-in-chief of the *Journal of Parkinson’s Disease* for 11 years and advises organizations like Cure Parkinson’s and The Michael J. Fox Foundation.

Day 2, Session 3

“Hot Topics” — The Future of Durable Therapies in Parkinson’s Disease

Joohi Jimenez, MD

Professor of Neurology and Medical Director
Mt Sinai Health System

Joohi Jimenez-Shahed, MD, is professor of neurology and medical director of Movement Disorders Neuromodulation and Brain Circuit Therapeutics at the Mount Sinai Health System. Her clinical and research work centers on deep brain stimulation, focused ultrasound and other neuromodulation approaches for Parkinson’s disease, dystonia, tremor and related movement disorders. She co-chairs the Parkinson Study Group’s Executive Committee and chairs its Healthcare Outcomes and Disparities Working Group. She also leads The Michael J. Fox Foundation-funded registry examining DBS outcomes. She earned her MD at Baylor College of Medicine, completed her neurology residency at Duke University Medical Center and her movement disorders fellowship at Baylor.

Richard Danzinger

Patient Voice

Closing Remarks

Sohini Chowdhury

Chief Program Officer
The Michael J. Fox Foundation

Day 2 Poster Session

A Dopamine Clock for Biological Staging of Parkinson's Disease

Roger Gunn, PhD

Chief Science Officer

Xing Imaging

Roger Gunn, PhD, is an international expert in imaging and drug development. He has discovered, developed and translated imaging techniques to measure biological processes for drug development, which are now central to biomarker strategies of pharmaceutical companies. As chief science officer at Xing Imaging, he leads the research and development of new biomarkers and analytics and oversees the design, analysis and delivery of clinical imaging trials for pharmaceutical companies. He is also emeritus professor of molecular neuroimaging at Imperial College and founder of the analytics software company MIAKAT Ltd. Dr. Gunn has published over 200 peer reviewed papers in the field of imaging, neuroscience and drug development with an H-index of 75 and has delivered over 80 invited lectures. His career has involved positions on research councils, consultancy to pharmaceutical companies and the training and mentoring of PhD students and clinical research fellows.

Abstract

BACKGROUND

Neuronal synuclein disease (NSD), characterized by aggregated α -synuclein followed by dopaminergic dysfunction, has been proposed as a biological definition of Parkinson's disease (PD) and related synucleinopathies. Current integrated biological and clinical staging approaches include qualitative biological measures. Latent time modeling may help quantify progression by reconstructing a canonical disease trajectory and estimating each participant's position along it, independent of when biomarkers were measured or diagnosis occurred.

OBJECTIVE

To develop a dopamine transporter (DAT) imaging framework for staging PD that quantifies disease progression using a latent disease-time model.

METHODS

DAT single-photon emission computed tomography (SPECT) data from more than 1,000 α -synuclein seed amplification assay (SAA)-positive participants in the Parkinson's Precision Medicine Initiative (PPMI) were analyzed using a latent time variable model. Putamen DAT binding was modeled as a logistic decline over disease time, with parameters estimated for the population and a time shift calculated for each participant. The model was also fitted to clinical measures, including the Unified Parkinson's Disease Rating Scale (UPDRS) Part III. Analyses explored the relationship between DAT-based stages and clinical measures, as well as trajectories across PPMI subgroups.

Day 2 Poster Session

A Dopamine Clock for Biological Staging of Parkinson's Disease (Continued)

RESULTS

The model identified a consistent logistic pattern of decline in putamen DAT binding and UPDRS Part III measures. Aligning individual participant trajectories to the canonical DAT curve enabled estimation of a subject-specific “dopamine clock,” representing biological disease time relative to the onset of measurable DAT loss. The model also supported a six-stage DAT-based system, from Stage 0 to Stage V. Higher DAT stage was associated with progressively higher UPDRS scores, supporting the potential utility of the clock and staging system for longitudinal assessment. Subgroup analyses suggested that participants with LRRK2 or GBA variants followed a similar temporal profile, with the modeled trajectory occurring approximately six years earlier on average.

CONCLUSION

Latent disease-time modeling of longitudinal DAT imaging reconstructs a canonical trajectory of dopaminergic decline in PD and enables estimation of an individualized dopamine clock. This framework may support quantitative biological staging, patient stratification and longitudinal monitoring of disease progression in research and clinical trials.

Day 2 Poster Session

Cognition Endpoints for Parkinson's Disease: A Roadmap for Developing Patient-focused Assessment Tools to Accelerate Drug Development

Chris Edgar, PhD

PCOA

Chris J. Edgar, PhD, is a psychologist and clinical outcomes expert with 26 years of experience in pharmaceutical drug development. His work focuses on clinical trial methodology, the selection and development of clinical outcome assessments (COAs), and the regulatory science needed to define meaningful endpoints across clinical development and product life cycles. He previously served as scientific director at Cognitive Drug Research Ltd., which developed the CDR System — one of the earliest computerized cognitive testing platforms widely used in clinical trials. His career also includes COA implementation work with Bracket/UBC, central nervous system (CNS) and neuroscience trial design and outcomes research at Roche and independent consultancy. Dr. Edgar is a member of the MJFF-supported cognition group, co-chair of the ISPOR Performance Outcome (PerfO) Assessment Task Force, and lead of the ECNP cognition subgroup.

Abstract

Cognitive changes are among the most worrisome symptoms associated with Parkinson's disease (PD). Treatments to address them remains an area of high unmet need because of their significant impact on daily function, interpersonal relationships and quality of life for people with PD (PWPD) and their care partners. PD dementia (PDD) represents a severe and disabling stage of the disease in which functional independence is lost. Importantly, there is substantial heterogeneity in the prevalence, severity, cognitive profile and rate of progression of cognitive dysfunction in PD. Estimates indicate 20–40% of PWPD have mild cognitive impairment (MCI) at an early stage or within two years of symptom onset, whilst overall prevalence has been estimated at 40%, with a life-long prevalence of 80% for dementia. Additionally, cognitive differences have also been observed in prodromal or at-risk groups, such as people with isolated REM sleep behavior disorder (iRBD). Rates of progression are variable, but a majority of PWPD surviving greater than 10 years disease duration will develop dementia, and those with GBA-PD are at risk of more rapid progression, while many of those in the early stages of PD may show little to no impairment or worsening in the near term. With the recent advances in biological definition and clinical staging of PD and dementia with Lewy bodies (DLB), DLB is also recognized as sharing both underlying neuropathology and aspects of clinical presentation, including prominent cognitive changes.

Due to the importance of cognitive dysfunction in PD, DLB and related disorders, since 2024 The Michael J. Fox Foundation (MJFF) has brought diverse stakeholders together, including academic, industry and non-profit representatives, to discuss the challenges and opportunities of outcome assessment and trial design for cognitive dysfunction in PD. This poster presents some of the proceedings of this dialogue and provides a roadmap for planned and ongoing activities supported by MJFF outlining priorities to advance patient-focused endpoints and improve clinical trial design to accelerate therapeutic development for cognitive dysfunction.

Day 2 Poster Session

Novel Modeling Approach for Comparing Treatment-naïve and Post-treatment Neuronal Synucleinopathy Progression Using Multi-modal PPMI Data

Piet Aarden, MSc

Director Data Science

Novartis Pharma

Piet Aarden, MSc, is director of data science at Novartis, where he serves as the late-stage development co-lead for The Michael J. Fox Foundation–Novartis collaboration. He leads a multidisciplinary team of statistical methodologists and data scientists focused on advancing disease understanding in neuronal synuclein disease. Since joining Novartis, Mr. Aarden has specialized in multimodal disease progression analysis, integrating diverse data sources to uncover insights into neurological disorders. He holds a master of science in mechanical engineering, with a specialization in control engineering, from Delft University of Technology.

Abstract

OBJECTIVES

To evaluate a novel multivariate progression-modeling framework in treatment-naïve and post-treatment periods in individuals with Neuronal alpha-Synuclein Disease (NSD).

BACKGROUND

Neuronal alpha-Synuclein Disease (NSD), a proposed nomenclature to designate people with evidence of neuronal-predominant alpha-synuclein aggregation with or without symptoms, begins years prior to clinical diagnosis of Parkinson's disease or Dementia with Lewy Bodies. Characterizing progression is challenging because disease stage and progression rate vary between individuals, and symptomatic treatment may alter clinical measures.

METHODS

Data were obtained from the Parkinson's Precision Medicine Initiative (PPMI). Individuals with NSD confirmed by positive CSF alpha-synuclein seed amplification assay were included. Six endpoints were analyzed: MDS-UPDRS Parts I, II, and III (Part III assessed OFF medication), SCOPA-AUT, and caudate and putamen DaT-SPECT striatal binding ratios. Consecutive-visit intervals were used to estimate endpoint-specific rates as functions of multivariate disease severity. We used 1:1 propensity-score matching on baseline endpoint values to compare untreated and post-treatment periods (298 pairs); follow-up was harmonized within pairs. Group progression curves were reconstructed from a common disease-severity anchor. Individual progression speed (α) was calculated relative to the modeled untreated rate at the same disease state.

Day 2 Poster Session

Novel Modeling Approach for Comparing Treatment-naïve and Post-treatment Neuronal Synucleinopathy Progression Using Multi-modal PPMI Data (Continued)

RESULTS

The analysis included 638 finite-difference intervals in each period after follow-up harmonization. At year 4, modeled MDS-UPDRS Part II scores were 5.0 in post-treatment versus 8.4 in untreated periods; Part III scores were 22.1 versus 31.1, respectively. α was lower in post-treatment periods for Parts II and III (Benjamini–Hochberg-adjusted $p=0.0009$ and $p=0.0002$). No evidence of slower progression was observed for caudate ($p=0.500$) or putamen ($p=0.950$) DaT-SPECT. Results are observational and do not establish a causal treatment effect.

CONCLUSIONS

In this matched case study, the framework detected the expected treatment-associated differences in MDS-UPDRS Parts II and III, while DaT-SPECT progression was similar between cohorts. These results support its potential use to quantify progression in observational datasets where symptomatic treatment starts at different times and may complicate interpretation of clinical outcomes. Potential applications include positioning subjects along a shared disease-evolution timeline, estimating progression speeds relative to the appropriate reference rate at their current disease state, identifying endpoint-specific or multivariate response patterns and helping interpret data from investigational treatment studies alongside observational datasets.

Notes

[illegible]



michaeljfox.org

