Worldwide collaborative framework for optimizing new Parkinson’s treatment trials with patient centric outcome measures

Diane Stephenson1, Yuge Xiao2, Catherine Kopil3, Karen Lee4, David T. Dexter5, Martijn Müller1, Klaus Romero1, Helen Matthews1, Gary Rafaloff6, Jodie Forbes7, Sarah Zener Dolen8, Carroll Siu9, Johan Hellsen10, John Crawford11, Mark Maybank12, Tanya Simuni12

1 Critical Path Institute, Tucson, AZ; 2 The Michael J. Fox Foundation, New York, NY; 3 Parkinson’s Canada, Ontario, Canada; 4 Parkinson’s UK, London, England; 5 Cure Parkinson’s; London, England; 6 Parkinson’s Patient/Researchers/Advocates, Northwestern University, Chicago, IL.

Background
• A rich pipeline of promising therapies is in development for Parkinson’s with a growing number targeting early intervention. (McFarthing et al., 2021)
• Historically, endpoints used in Parkinson’s trials have relied on clinician-based outcome assessments and/or in-clinic performance outcome (PerFO) assessments for example, UPDRS.
• The FDA is driving a new era of Patient-Focused Drug Development, requiring trials to use patient-centered endpoints.
• Multi-stakeholder collaborations are needed to develop new patient reported outcome measures for Parkinson’s.
• A consensus roundtable focused on endpoints was held in Washington DC, November 2-3, 2022.

Methods
• A diverse array of stakeholders from around the world were engaged as active participants in the workshop.
• Precompetitive collaboration and willingness to share regulatory insights are key requirements for all roundtable participants, including contributing organizations.
• A panel of people with lived experience with Parkinson’s led a dynamic panel discussion.
• Complementary to ongoing initiatives: CPP endpoints, MJFF advisory council, DHT efforts.

Results
• Reviewed a total of 8 case studies at Nov. 2-3 meeting and within the C-Path's Patient-Centric Outcomes Team.
• FDA issues call to action for new biological staging framework.
• Published November workshop proceedings report (open access):

Actions and next steps
• Develop a consensus conceptual model for [early] Parkinson’s from qualitative data.
• Expand CPP’s 3DT effort to centralize intelligence on digital metrics in development for Parkinson’s and expand the list of publicly available information.
• Develop Parkinson’s Concepts of Interest (COI) item bank.
• Maintain ongoing forums: CPP’s Patient-Centric Outcomes Team & MJFF’s Endpoints Advisory Council.

Efforts Underway
An array of different approaches for better endpoints are being advanced by experts across the globe

• Industry led efforts tied to drug trials.
• Academic led research initiatives.
• Nonprofit driven initiatives and research.
• Movement Disorder Society outcomes expert led program.
• Global strategies via Public Private Partnerships in the area of digital health technologies.

Implementing a biological staging system accelerates progress, regardless of the approach taken.

You can help by participating in research:
• Fox Trial Finder: https://www.michaeljfox.org/trial-finder
• Fox Insight Online Study: https://foxinsight.michaeljfox.org/
• Buddy Network: study postings in the “Research” group board:
https://research.michaeljfox.org/