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May 19, 2016

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- Consortium, led by Parkinson's UK and the Critical Path Institute, aggregates and analyzes study data to develop a model for quantitative characterization of Parkinson's progression
- This asset would enrich the design of trials for novel therapies
- Consortium will work with international regulators for acceptance of a quantitative model to streamline review processes

NEW YORK, LONDON AND TUCSON (May 18, 2016) — The Michael J. Fox Foundation for Parkinson's Research (MJFF), with Parkinson's UK and the Critical Path Institute (C-Path) in Tucson, Arizona, announce that MJFF has joined the Critical Path for Parkinson's Consortium (CPP). The consortium aims to create a quantitative model of Parkinson's progression from its earliest stages that will allow researchers to optimize clinical trial design for faster and more effective testing of novel therapies.

Parkinson's UK and C-Path launched the CPP in October 2015, and seven major pharmaceutical companies, the Parkinson's Disease Foundation, the Davis Phinney Foundation and Cure Parkinson's Trust have subsequently signed on to the initiative.

"More therapies with potential to slow or stop Parkinson's progression in its early stages are moving through clinical trials," said MJFF Senior Vice President of Research Programs Mark Frasier, PhD. "A quantitative characterization of that early progression would help evaluate the impact of these treatments and usher effective therapies to the people who need them."

Currently clinical trials for potential new therapies are time-consuming and costly. Due to the variability in Parkinson's symptoms and progression and the lack of objective biomarkers, trials must enroll hundreds of patients and follow them for many months if not years. Development of a quantitative model of Parkinson's progression, potentially combining both biological and clinical factors, may allow for efficient trials that aim

to treat the right patient with the right drug at the right time.

To create the model, C-Path will standardize and aggregate data from several large-scale Parkinson's studies — including the MJFF-led Parkinson's Progression Markers Initiative (PPMI) comprising nearly 1,000 participants, which has made its data available in real time since it launched in 2010. C-Path will apply modeling and computational tools using the pooled database to produce the quantitative progression model — which will be submitted to the U.S. Food and Drug Administration and the European Medicines Agency for feedback — with the ultimate goals of saving time and money and providing a platform for the best chance of a positive outcome.

In future stages, the consortium plans to develop a clinical trial simulation platform. Drug developers make educated guesses on the number of volunteers to enroll, how long to follow them and how to evaluate therapeutic impact. The simulation platform will inform trial sponsors of the required parameters when planning a trial and allow them to enter data on individual participants to determine the number of subjects and the length of the trial required. For example, with a study population of "fast progressers" as defined by the CPP progression model, sponsors may be able to plan a shorter trial. Such a tool would help drug developers plan efficient studies and avoid false negative results.

"We are very pleased to welcome The Michael J. Fox Foundation to this important collaboration," said Martha A. Brumfield, president and CEO of C-Path. "The Foundation's unwavering commitment to helping patients with PD, their years of experience in articulating the patient perspective, and their research priorities will enrich the work of the Critical Path for Parkinson's Consortium."

Arthur Roach, PhD, director of research at Parkinson's UK, the principal funder of the consortium, says: "Parkinson's UK is determined to get better treatments to people living with this devastating condition in years, not decades. A key part of this is joining forces with regulators, researchers, pharma and medical research charities such as MJFF to maximize the benefit of data-sharing and learnings. Working with CPP partners will play a crucial role in helping develop and evaluate the effectiveness of potential new therapies, which could one day lead us to a cure for Parkinson's."

The PD data platform will serve as the resource for advancing the regulatory science goals of CPP, including qualification of drug development tools, and eventually will be made available to qualified researchers. Through CPP analyses or independent investigation, the standardized, aggregated data may allow researchers to identify events that underlie trajectories of decline and biomarkers or other determinants of early Parkinson's disease.

About the organizations:

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 improved 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 more than $600 million 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; 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. For more information, visit us on the Web, Facebook, Twitter, and LinkedIn.

Every hour, someone in the UK is told they have Parkinson's. It affects 127,000 people in the UK, which is around one in 500 of the population. Parkinson's is a degenerative neurological condition, for which there currently is no cure. The main symptoms of the condition are tremor, slowness of movement and rigidity. Parkinson's UK is the UK's leading charity supporting those with the condition. Its mission is to find a cure and improve life for everyone affected by Parkinson's through cutting edge research, information, support and campaigning. For advice, information and support, visit www.parkinsons.org.uk.

Established in 2005 as a nonprofit organization, C-Path was formed to deliver on the vision of FDA's Critical Path Initiative. C-Path is committed to improving human health and well-being by developing new technologies and methods to accelerate the development and review of medical products. An international leader in forming collaborations around this mission, C-Path has established twelve global, public-private partnerships that include more than 1,400 scientists from regulatory and other government agencies, academia, patient advocacy organizations, and 41 major pharmaceutical companies. C-Path has headquarters in Tucson, AZ, and has been funded by public and private philanthropic support from the University of Arizona, Science Foundation Arizona (SFAz), the US Food and Drug Administration (FDA), and the Tucson community. For more information, visit www.c-path.org.

C-Path Contact:
Kissy Black
+1.615.298.1144
kissyblack@lotosnile.com