

## FALL 2020 FUNDING PROGRAM PARKINSON'S PATHWAY BIOMARKERS

### BACKGROUND

Parkinson's disease (PD) affects nearly 1 million people in the US and over 6 million worldwide, and those numbers are expected to rise over the coming decades. PD is highly heterogeneous: individuals experience a wide array of motor and non-motor symptoms, many of which depend on disease severity and duration. Though our understanding of PD and its causes is growing, many questions remain. Currently, there are no disease-modifying drugs available for Parkinson's while standard symptomatic treatments provide some limited relief but come with complications and side effects.

The Michael J. Fox Foundation (MJFF) funds research to better define, measure, and treat Parkinson's disease as well as critical tools and other resources to advance that research. The purpose of this Request for Applications (RFA) is to develop biomarkers that can support therapeutic development for priority pathways in PD. Specifically, the development of biomarkers for pathways that are known to be affected in PD and are the focus of therapeutic efforts—such as inflammation, mitochondrial function, lysosomal function, autophagy, protein trafficking, and protein handling—would dramatically improve our ability to diagnosis, objectively track the progression of disease, enrich for subject populations, or more precisely detect a treatment response or target engagement. The availability of all these types of biomarkers would transform clinical trial design and assist in the interpretation of the results.

### DEADLINES & REVIEW SCHEDULE

- Pre-proposals Due: April 23, 5p.m. EST
- Full Proposal Invitations: June 11, 2020
- Full Proposals Due (by invite only): August 11, 5p.m. EST
- Anticipated Award Announcement: November 2020
- Anticipated Funding: December 2020

*Applicants are encouraged to apply early to allow adequate time to correct errors found during the submission process.*

### FUNDING AVAILABLE

**Duration:** One- to-two-year grants.

**Award Amount:** Up to \$300,000. Requested support should be commensurate with work proposed. These budgets include direct and indirect costs. MJFF policy is that no more than 15% (non-profit institutions) or 10% (for-profit organizations) of the budget may go to indirect costs. Additional details about MJFF's indirect cost policy can be found in the [Application Guidelines](#) and [FAQ](#).

### ELIGIBILITY REQUIREMENTS

Applications may be submitted by researchers or clinicians in:

- U.S. and non-U.S. biotechnology/pharmaceutical companies, or other publicly or privately held for-profit entities; and
- U.S. and non-U.S. public and private non-profit entities, such as universities, colleges, hospitals, laboratories, units of state and local governments and eligible agencies of the federal government.
- Post-doctoral fellows are **not** eligible to apply as principal investigators to this program.

## PROGRAM GOAL

Ultimately, our goal is to develop better biomarkers for PD diagnosis, disease progression, patient stratification, and/or pharmacodynamic readouts for clinical trials. Currently, many efforts are focusing on understanding the role of various cellular processes/pathways in PD and uncovering novel targets within these pathways for therapeutic development. **As such, sensitive biomarker readouts for these targets and pathways will be critical for bridging the gap from basic to translational research/clinical trials.** To accelerate therapeutic development through the development of companion outcome measures/assays, we are launching this RFA to support critical biomarker development aimed at (1) sensitive measurements of pathway activation/dysfunction and (2) improving technologies for analyzing the target/pathway of interest.

MJFF considers priority pathways to include: mitochondrial function, lysosomal function, autophagy, inflammation, protein handling, protein trafficking, etc.

## PROGRAM PRIORITIES

Applications should focus on studies that achieve one or more of the following goals:

- Develop sensitive target or pathway-based outcome measures/assays for PD to aid diagnostic efforts, tracking disease progression, disease subtyping, patient stratification, pharmacodynamic readouts, or determination of therapeutic efficacy. Novel assay development could include:
  - Developing outcome measures/assays to measure targets that are sensitive and specific for assessing PD-affected pathways.
  - Developing/expanding multiplexed biochemical assays to assess multiple readouts of pathway function/dysfunction in PD.
- Optimize existing outcome measures/assays to assess PD-related pathways or pathway-based targets. Optimization efforts could include:
  - Testing existing outcome measures/assays developed for other disease areas in PD to assist with patient stratification, assessing target/pathway engagement for clinical trials, or measuring efficacy of a PD therapeutic intervention.
  - Adaptation of existing assays to different biological matrices to reduce invasiveness of the assay or improve assay sensitivity/specificity.
  - Validating promising assays across various performance parameters (e.g., robustness, precision, trueness).
  - Reformatting promising assays to ensure they use sustainable/renewable resources or bridging home-brew assays to the commercial space to ensure open accessibility.
- Work in preclinical models and human biospecimens to develop translational biomarkers that can be assessed in Parkinson's disease models as well as patient biospecimens using the same matrix and same assay technologies.

Please note:

- Applicants should indicate if the proposed methodology has been applied in human biosamples, noting use in samples from patients with PD or other neurodegenerative diseases.
- Studies interested in discovery work identifying new targets in patient biosamples or validating the PD-relevance of targets identified in preclinical models should apply to the open MJFF Target Advancement Program (more information on the Funding Opportunities webpage). Proposals for this callout should focus on the development of biomarkers for pathways/targets with established relevance to PD.

## BIOSAMPLE REQUESTS

Investigators are encouraged to leverage existing tissue and biosample resources if possible. Studies requesting access to biosamples available through MJFF-sponsored biospecimen collections are eligible to apply to this initiative. In these cases, access to samples will be reviewed in parallel to funding requests by the committees overseeing the biospecimen collection(s) requested. Access to samples will not guarantee funding, and funding will not guarantee access to samples. To review MJFF's biosample collections, please consult the MJFF biorepository [website](#) and [biorepository inventory catalogue](#).

Groups requesting access to samples only (without funding) should contact [resources@michaelfox.org](mailto:resources@michaelfox.org) for information on how to request samples.

## ADDITIONAL INFORMATION

Our [Application Guidelines](#) provide general guidance about applying for funding from MJFF, though the RFA always supersedes information contained in the Application Guidelines. Please note that MJFF updated our publication and indirect costs policies in early 2020. The new [open access publication policy](#) requires articles resulting from MJFF-funded work publish in a preprint repository then in an open access forum with free and immediate readership rights.

MJFF will host an informational webinar on March 27, 2020 at 12 p.m. ET to clarify and explain the goals of our funding opportunities and answer applicant questions. The webinar will be available to view on-demand after the live airdate. To register, please visit the [Parkinson's Pathway Biomarkers](#) webpage.