THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

Fall 2019 Core Funding Programs
Therapeutic Pipeline Program
An Edmond J. Safra Core Program for PD Research

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 support the development of new treatments and interventions with potential for significant impact for people with PD.

PROGRAM GOAL
The Therapeutic Pipeline Program (TPP) seeks to speed the development of Parkinson's disease therapeutics with potential for fundamentally altering disease course, significantly improving treatment or management of symptoms beyond current standards of care. Proposals should focus on strategies with clear impact for people with PD and well-defined development plans, which can include traditional pharmacological treatments, biological therapies, gene therapy, surgical, non-invasive/non-pharmaceutical or applied technology approaches. Both novel programs and repurposing of approved or clinically safe therapies from other disease indications are appropriate for this RFA. Applied technologies that have been preliminarily tested in other neurological conditions but have not been tested in Parkinson's are also suitable for this program. MJFF will prioritize approaches built on strong scientific and biological rationale for targeting fundamental processes underlying PD cause, progression and/or symptom expression. For applied technologies, projects seeking to address major symptomatic or quality-of-life challenges for people with Parkinson's are ideal.

Applications must address one of the following treatment challenges:
- Protection or restoration of degenerating and/or dysfunctional neurons affected in PD
- Assist with minimizing or reducing impact of symptoms not well managed by current treatments
- Reduction of complications and side effects of current PD treatments.

PROGRAM PRIORITIES
Applicants may apply to any of the following three tracks.
The **TPP Pre-clinical Track** supports work in the following areas:
- Assay development, early screening campaigns and therapeutic optimization to identify approaches with ability to selectively target disease features and mechanisms with strong and compelling rationale for PD,
- Proof-of-concept studies validating the potential benefits of a therapeutic strategy in pre-clinical models of PD and/or PD-relevant pathogenic mechanisms, and

- Characterization of promising therapeutic approaches to obtain data on PD-relevant pharmacokinetics, pharmacodynamics, safety and other features critical for progress into future clinical testing stages.

The **TPP Clinical Track** supports work in the following areas:

- Phase I trials including first-in-human, pharmacokinetic/pharmacodynamic and early safety/tolerability studies.
- Phase II trials seeking early proof-of-concept clinical and biological efficacy data, and
- Phase III trials seeking pivotal demonstration of clinical efficacy.

NEW Program Track: **TPP-Applied Technologies as Treatments** supports work in the following areas:

- Studies seeking to collect proof-of-concept efficacy and/or feasibility data from prototype or available devices and technologies designed to directly address critical, poorly managed symptoms of PD or assist individuals with PD to perform daily tasks with less difficulty.
- Analysis of existing clinical datasets from studies of applied technologies to look for evidence of clinical impact in people with PD
- Note: Proposals to test remote monitoring devices that do not have a treatment focus (e.g. wearable activity trackers) or general exercise and rehabilitation approaches are not appropriate for this program track.

Successful applications will provide strong and compelling data supporting the biological and clinical rationale for the proposed treatment and a clear plan, including essential "Go/No-Go" decision milestones, for moving the approach through the essential stages of development. Investigators new to PD are encouraged to collaborate with experienced PD scientists, clinicians and/or industry.

For clinical programs, given higher budgetary needs, MJFF funding should ideally leverage other funding sources with MJFF support focusing on activities that can, for example, support collection of critical PD-relevant outcome measures, targeted recruitment needs and/or other challenges that can help ensure faster and more PD-informative results. Please also note that MJFF prefers to work closely with study sponsors to ensure trial protocols and plans are designed and executed with the greatest chance for success. Moreover, MJFF looks for studies that have developed a thorough recruitment and retention plan to meet the study goals on time. For further information, please consult our available recruitment and retention resources at Trial Resource Pack.

DEADLINES & REVIEW

- Pre-proposals Due: April 19th, 2019
- Full Proposal Invitations: Week of June 10th, 2019
- Full Proposals Due (by invite only): July 19th, 2019
- Anticipated Award Announcement: September 2019
- Anticipated Funding: October 2019

Applicants are encouraged to apply early at the pre-proposal stage to allow adequate time for resubmission if errors are found during administrative review.

FUNDING AVAILABLE

Duration: One- to two-year grants for preclinical and applied technology as treatment programs; two- to three-year grants for clinical programs.

Award Amount:

- Up to $500,000 for Pre-clinical Program
- Up to $500,000 for Applied Technology Program
- Up to $2,000,000 for Clinical Program

Final budgets will be determined based on review of proposed work and MJFF role. MJFF may not be able to support all costs for a proposed therapeutic development plan and applicants are encouraged to leverage additional sources of funding and resources. These budgets include direct and indirect costs. For academic and for-profit institutions, no more than 25% or 10%, respectively, may go to indirect costs. Additional details about MJFF's indirect cost policy can be found in the Administrative 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.

As therapeutic programs may require many kinds of expertise, MJFF encourages industry and academic collaborations when appropriate.

ADDITIONAL INFORMATION

Our Administrative Guidelines provide general guidance about applying for funding from MJFF. Please note that the RFA always supersedes information contained in the Administrative Guidelines.

MJFF will host an informational webinar on April 1, 2019 at 12 p.m. ET to clarify and explain the goals of MJFF funding opportunities and answer applicant questions. The webinar will be available to view on-demand after the live airdate. To register, please visit the Therapeutic Pipeline Program webpage.