

## SPRING 2018 CORE FUNDING PROGRAMS

### THERAPEUTIC PIPELINE PROGRAM: *AN EDMOND J. SAFRA CORE PROGRAM FOR PD RESEARCH*

#### PROGRAM GOAL

The Michael J. Fox Foundation for Parkinson's Research (MJFF) seeks to speed the development of Parkinson's disease (PD) therapeutics with potential for fundamentally altering disease course and/or significantly improving treatment of symptoms beyond current standards of care. The Therapeutic Pipeline Program (TPP) is open to industry and academic investigators working at pre-clinical or clinical stages of drug development. Proposals should focus on treatment strategies with clear impacts for people with PD and well-defined development plans. Both novel approaches, and repurposing of approved or clinically safe therapies from other disease indications are of interest for this program.

#### DEADLINES

Spring 2018 Review Cycle

- Pre-proposals Due: September 27, 2017 – 5 p.m. US ET
- Full Proposal Invitations: December 2017
- Full Proposals Due (by invite only): January 23, 2018 – 5 p.m. US ET
- Anticipated Award Announcement: April 2018
- Anticipated Funding: May 2018

#### BACKGROUND

PD is a progressive neurodegenerative disease affecting more than five million people worldwide, with significant prevalence growth expected due to an aging population. Current therapies address mild-to-moderate motor symptoms of the disease but lose effectiveness over time and are associated with challenging long-term side effects. There are limited drugs targeting non-motor aspects of the disease, and, no current treatment can slow or stop the underlying degenerative process.

The path from research discovery to approval of a promising new treatment is long and often uncertain. New ideas about PD cause and progression lead scientists to propose potential therapeutic strategies. Translation of these approaches into clinical significance for patients requires extensive work along a critical pipeline from early target biology validation to pre-clinical drug development, and ultimately to human clinical testing. Keeping the pipeline filled with promising ideas is paramount to ensuring development of new treatments for people with PD.

The core mission of MJFF is to accelerate drug development for PD. Innovative strategies, such as our annual Core Funding Programs, including The Edmond J. Safra Core Programs for PD Research, identify roadblocks along the therapeutic development pipeline and share risks with academic and industry researchers. Through these and other efforts, we hope to increase the movement of new treatments to the clinic.

## PURPOSE

TPP supports PD therapeutic development at both pre-clinical and clinical stages. The program is open to any creative strategy seeking to address one of the following treatment challenges:

- Protection or restoration of degenerating and/or dysfunctional neurons affected in PD
- Alleviation of disabling motor or non-motor symptoms of PD not well managed by current treatments, or
- Reduction of complications and side effects of current PD treatments.

Therapeutic strategies with clear potential impact for patients, including traditional pharmacological treatments, biological therapies, gene therapy, surgical and non-invasive/non-pharmaceutical approaches, are all appropriate for this program. However, MJFF will prioritize approaches built on strong biological rationale for targeting fundamental processes underlying PD cause, progression and/or symptom expression. As similar biology and therapeutic approaches may be shared across different diseases, MJFF is also interested in repositioning treatments for PD that have been approved or shown to be clinically safe for other indications.

## PROGRAM DETAILS

Applicants may apply to one of two tracks based on stage of therapeutic development.

- 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 where MJFF support can leverage other sponsor funding sources to expand critical data collection, explore PD-relevant biomarker analyses and outcome measures, or support targeted recruitment needs.

Successful proposals will provide strong and compelling data supporting rationale for the proposed therapeutic 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 to ensure proposals have the greatest chance for consideration.

For clinical programs in particular, if funded, please note that MJFF prefers to work closely with sponsors to ensure trial protocols and plans are designed and executed with the greatest chance for success. In addition to potential funding, MJFF provides opportunities to work with key stakeholders to develop patient recruitment and retention strategies, optimize clinical operations, engage patients, and understand regulatory issues and/or other barriers impeding progress in PD drug development. Please contact [trialsupport@michaeljfox.org](mailto:trialsupport@michaeljfox.org) to discuss how MJFF can best support your clinical development program.

## FUNDING AVAILABLE

MJFF will provide the following funding to this program:

- **TPP Pre-clinical Track** awards range from \$100,000 to \$500,000 for one-to-two year programs.
- **TPP Clinical Track** awards up to \$2,000,000 for two-to-three year programs.

MJFF reserves the right to reduce the duration and budget of awarded proposals based on their scope, review and alignment with MJFF's priorities. As the cost of drug development can be significant, MJFF may not be in a position to support all costs for a proposed therapeutic development plan. Applicants are encouraged to leverage additional sources of funding and resources where MJFF support may accelerate progress and expand the rigor and robustness of data supporting a therapeutic strategy. Although not guaranteed, MJFF may offer opportunities for supplemental funding based on strong continued and demonstrated promise of a therapeutic approach for PD.

Please note that for academic and for-profit institutions, no more than 25% or 10%, respectively, may go to indirect costs. More details about our indirect cost policy can be found in [Administrative Guidelines](#) and our [FAQ](#).

All funding from MJFF is non-dilutive although in certain cases MJFF may request defined multiple grant repayments based on achievement of future commercial milestones.

## ELIGIBILITY REQUIREMENTS

Applications may be submitted by:

- 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.

As therapeutic programs may require many kinds of expertise, MJFF encourages industry and academic collaborations when appropriate. Given the significant coordination and leadership necessary for TPP, post-doctoral fellows are **not** eligible to apply as PIs.

## ADMINISTRATIVE GUIDELINES

Please refer to our [Administrative Guidelines](#). Please note that any information contained in the above program description and additional program instructions will supersede any information contained in general MJFF administrative guidelines.

**FURTHER INFORMATION**

MJFF will host an informational webinar to clarify and explain the goals of MJFF funding opportunities and answer applicant questions. To register, please visit the [Therapeutic Pipeline Program](#) webpage.