

Fall 2022 Request for Applications Translational Pipeline Program



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. There are no drugs available for Parkinson's that alter the progression of the disease, and current symptomatic treatments provide 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 translate research into the investigation and development of therapies with potential for significant impact for people with Parkinson's disease.



PROGRAM GOAL

The Translational Pipeline Program seeks to foster and accelerate the development of Parkinson's disease therapeutics with potential for fundamentally altering disease course or significantly improving treatment or management of non-motor or motor symptoms beyond current standards of care.

Funding will support projects that aim to:

- Advance early-stage targets with preliminary rationale for PD pathophysiology and/or symptomology into preclinical therapeutic development by using potent and selective tool compounds to demonstrate therapeutic potential.
- Advance through critical preclinical stages the identification, validation, or development of novel approaches and/or new treatments or interventions with potential for significant impact for people with Parkinson's.

- Repurpose or reposition approved or clinically safe therapies from other disease indications.
- Progress promising interventions into initial Phase I trials to assess pharmacokinetic/pharmacodynamic and early safety/tolerability.

For this round, MJFF **will not consider** proposals focused on the following:

- Applications proposing early discovery studies to identify new targets (e.g., large-scale screening or genomic/transcriptomic analyses).
- Applications proposing genetic model systems and/or genetic manipulation to validate biological targets, except if used as therapeutic approach(es).



PROGRAM PRIORITIES

When considering applications submitted to this program, MJFF prioritizes those that:

- Aim to validate biological targets for which human genetic or clinical data linking the target to PD exists or preliminary data showing altered expression or function of the target in PD-relevant human tissue or biofluids.
- Propose use of potent and selective pharmacological tools to validate early-stage targets. Mechanisms of validation of well-known targets, such as alpha-synuclein, LRRK2, GBA and Parkin/PINK1, will be considered only when novel approaches of targeting with significant therapeutic value are being proposed.
- Develop primary assays, early screening campaigns, hit-to-lead and lead optimization studies with the purpose of identifying approaches to selectively target disease features and mechanisms with strong and compelling rationale for PD.
- Propose proof-of-concept studies to validate the potential benefits of a therapeutic strategy in pre-clinical models of PD and/or PD-relevant pathogenic mechanisms, and that integrate a comprehensive plan to evaluate pathway-relevant biomarkers of both target and therapy engagement.
- Aim to characterize promising therapeutic approaches by obtaining pharmacokinetics, pharmacodynamics, safety and other data critical for progress into future clinical testing stages.

This cycle, MJFF is particularly interested in:

Efforts focused on identifying and/or developing therapies and/or approaches to address pressing, unmet patient need in managing key symptoms including:

- Moderate-to-advanced disease motor symptoms such as gait disorder, balance issues, falling, freezing, and postural instability.

- Non-motor disease symptoms such as cognitive changes, sleep disturbances, pain, fatigue, and mood disorders including anxiety, depression, and apathy.
- Autonomic dysfunction difficulty including orthostatic hypotension, dysphagia, sialorrhea, gastroparesis, constipation, dysarthria, bladder dysfunction, and sexual dysfunction.



FUNDING AVAILABLE

Duration:

- 1 to 2 years for pre-clinical program (target validation and therapeutic development)
- 2 to 3 years for clinical program (Phase I clinical trials)

Award Amount: \$250,000 - \$2,000,000

MJFF has a flexible approach to funding translational work that is guided by the novelty of the approach, the stage of development and the overall priority of the therapeutic need for people with PD. Along these lines, award amounts for this program may range from \$250,000 for smaller, targeted programs to \$2M for larger, multi-stage preclinical and/or clinical programs. Higher awards (e.g., up to \$5M) may be offered at the discretion of MJFF based on strong need and justification. Applicants will be given guidance on appropriate budget consideration if invited to the full proposal stage.

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 15% or 10%, respectively, may go to indirect costs. Additional details about MJFF's indirect cost policy can be found in the [Application Guidelines](#) and [FAQ](#).



DEADLINES & REVIEW SCHEDULE

- Pre-proposals Due: April 26, 2022, 5 p.m. US ET
- Full Proposal Invitations: Week of June 6, 2022
- Full Proposals Due (by invite only): August 11, 2022, 5 p.m. US ET
- Anticipated Award Announcement: Week of November 14, 2022
- Anticipated Funding: November 2022

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



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 or Co-Principal Investigators



BIOSAMPLE REQUESTS

As part of the study, applicants may propose to validate a biomarker assay utilizing a tool compound or clinical candidate using PD biosamples. Investigators are encouraged to leverage existing tissue and biosample resources if possible. New recruitment should be limited to studies where clinical biosamples are unavailable for the intended purpose (ex. different collection protocol required, specific cohort required, or novel biomatrix proposed). Studies requesting access to biosamples available through MJFF-sponsored biospecimen and cell line 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. To review MJFF's available biosample collections, please consult the MJFF biorepository [website](#) and [biorepository inventory catalogue](#). Groups requesting access to samples only (without funding) should contact resources@michaeljfox.org.



DIVERSITY, EQUITY AND INCLUSION (DEI)

In pursuit of our mission to accelerate the development of better treatments and a cure for Parkinson's disease, MJFF aims to support a rigorous research agenda reflecting a wide and diverse range of perspectives on Parkinson's disease and carried out in diverse populations. Diversity may refer to characteristics including, but not limited to, race, religion, ethnicity, sex, gender identity, sexual orientation, socioeconomic circumstance, nationality, geographic background, ability and disability, political ideology and age. Parkinson's is a complex problem; the more angles from which we attack, the greater the chances of finding innovative scientific solutions to benefit everyone living with the disease. As such:

- The Foundation encourages applications from diverse investigators representing groups historically underrepresented in the research enterprise.
- Because research shows that diverse teams outperform homogeneous ones, we urge applicants to share information about the composition of the team that will carry out the funded work.
- Applicants should consider how to incorporate diversity in the research methodology of the proposed studies (e.g. more diverse patient populations in clinical trials, or more diverse sample selection in pre-clinical studies).



ADDITIONAL INFORMATION

The [Application Guidelines](#) provide general guidance on applying for funding from MJFF, though the RFA always supersedes information contained in the Application Guidelines.

MJFF holds an [open access publication policy](#) requiring articles resulting from MJFF-funded work to be published in a preprint repository, then in an open access forum with free and immediate readership rights.

MJFF requires that the Principal Investigator be the primary applicant (i.e., the person who initiates and takes primary responsibility for the application). All application-related correspondence will be sent to the Principal Investigator.

For questions about the application process or project suitability for this call for applications, please email grants@michaeljfox.org.