

## SPRING 2022 REQUEST FOR APPLICATIONS TARGET ADVANCEMENT 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. 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 basic research and develop tools to enable therapeutic development. To this end, MJFF believes that promoting early-stage target validation and tool development studies within academic and industry laboratories can bridge the critical gap between basic biology and therapeutic development.

### DEADLINES & REVIEW SCHEDULE

- Pre-proposals Due: September 28, 2021, 5 p.m. US ET
- Full Proposal Invitations: Week of November 15, 2021
- Full Proposals Due (by invite only): January 13, 2022, 5 p.m. US ET
- Anticipated Award Announcement: Week of April 18, 2022
- Anticipated Funding: April 2022

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

### FUNDING AVAILABLE

**Duration:** 18 to 24 months

**Award Amount:** Up to \$200,000. Requested support should be commensurate with work proposed. 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](#).

### 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 eligible to apply as co-investigators with the designation of an administrative primary investigator who directs the laboratory in which the fellow will conduct research. The administrative co-PI will be responsible for assisting in providing all institutional documents required for the project and will be required to sign any award contract. Training or mentoring-only proposals will not be considered.

#### PROGRAM GOAL

The Target Advancement Program seeks to build conclusive evidence around and develop field-enabling tools for early-stage biological targets in Parkinson's Disease (PD) in order to enable the initiation of future preclinical therapeutic programs focused on those targets. Funding will support projects to validate early-stage targets previously implicated in PD pathophysiology and/or symptomology but that have not yet been rigorously investigated for their ability to impact disease-relevant outcomes in PD model systems. For targets that are lacking preclinical tools and/or models required for validation, the Tool Generation track provides funding for the development of research-enabling preclinical tools and models to assist with the validation of the selected target.

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

- Validation of well-known targets, including alpha-synuclein, LRRK2, GBA and Parkin/PINK1 given significant current investments MJFF is already making in these areas.
- Applications proposing discovery studies to identify new targets (e.g., large-scale screening or genomic/transcriptomic analyses).

#### PROGRAM PRIORITIES

The **Tool Generation for Target Validation track** supports:

- The development of a research-enabling preclinical tool (ie rodent model, antibody, cell line, etc), basic characterization of the novel tool, and use of the tool in a study investigating the mechanistic link of the target to PD (following the same criteria as the "Target Validation Program").
- Projects must focus on developing tools for and performing validation of targets that are novel/under-investigated in PD. Applications focusing on development of tools where validated alternatives exist (ie antibodies to aggregated forms of alpha-synuclein, development of preclinical models overexpressing wildtype human LRRK2, etc) will not be considered.
- Working with MJFF to deposit the resulting tool in an MJFF-designated repository for community access is a requirement of the program.

The **Target Validation track** supports:

- Projects that demonstrate that genetic/pharmacological modulation of target expression/activity in in vivo mammalian models affects biological pathways relevant to PD pathogenesis and yields outcomes predictive of therapeutic efficacy.
- Replication or further validation studies of recently published, high-impact findings in PD target validation biology that could increase confidence in starting new therapeutic translational programs.
- Proposals may include but should not be limited to the confirmation of the relevance of an early-stage target by studying its altered expression, distribution, or pathological function in human PD tissues.

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

- Have existing human genetic or clinical data linking the target to PD or that have preliminary data showing altered expression or function of the target in PD-relevant human tissue or biofluids.
- Emphasize use of established experimental model systems with high construct validity for human PD, such as exhibiting clear, measurable altered expression or function of the target and/or mechanism of interest.
- Propose use of established methods for genetic or pharmacological modulation of the target in experimental model systems, including demonstration that methods are specific and capable of engaging the target in the tissue of interest.

#### **BIOSAMPLE REQUESTS (TARGET VALIDATION PROGRAM ONLY)**

Applicants may propose studies designed to validate PD targets in pre-clinical models, human cellular models, and human tissue and fluids. Investigators are encouraged to leverage existing tissue and biosample resources if possible. 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](mailto:resources@michaeljfox.org).

#### **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.

Please note, MJFF now 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.

#### **DIVERSITY, EQUITY AND INCLUSION**

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.

- All applicants are encouraged to provide information about the steps they will take to carry out their investigations in diverse clinical populations.

#### INFORMATIONAL WEBINAR

MJFF will host an informational webinar on August 24<sup>th</sup>, 2021, 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. Please register [here](#).

For questions about the application process or project suitability for this call for applications, please email [grants@michaeljfox.org](mailto:grants@michaeljfox.org).