THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

2017 MJFF FUNDING PROGRAM
INFLAMMATION BIOMARKERS FOR PARKINSON'S DISEASE

PROGRAM GOAL

The Michael J. Fox Foundation for Parkinson's Research (MJFF) seeks to support research that will develop new or improve upon existing biomarker tools for neuroinflammation/peripheral inflammation in Parkinson's disease (PD). The specific goals of this initiative are to: 1) facilitate the development of biomarker measures that can be utilized for novel target validation, 2) assess pathological progression or phenoconversion, or 3) identify inflammation paradigms unique to PD. Proposed biomarkers should facilitate objective decisions to validate a particular target or target efficacy marker (downstream readouts of successful target modification), improve the ability to enrich subject populations in clinical trials and/or determine whether experimental treatments are modifying the course of the disease, its symptoms or its progression.

DEADLINES

Fall 2017 Review Cycle

- Pre-Proposals Due: May 31, 2017 – 5pm US ET
- Full Proposal Invitations: June 30, 2017
- Full Proposals Due (by invite only): August 4, 2017 – 5pm US ET
- Anticipated Award Announcement: October 2017
- Anticipated Funding: November 2017

BACKGROUND

Parkinson's disease is a heterogeneous disorder and a host of emerging targets may provide useful new therapeutic development opportunities. Neuroinflammation/inflammation are increasingly recognized as components of PD, but their role in PD etiology and progression remains poorly understood. In order to better identify the specific role for neuroinflammation/inflammation in PD and to advance promising immunomodulatory targets through the therapeutic pipeline, well validated neuroinflammation/peripheral inflammation biomarkers are critically needed. Additionally, a successful therapeutic clinical trial program for PD is dependent on access to robust, informative tools to assist in patient selection/stratification, interpretation of the biologic impact of therapy, and determination of medically relevant therapeutic efficacy in early phase trials. For all of these reasons, MJFF seeks to sponsor research studies aimed at elucidating PD-specific biomarkers for neuroinflammation/peripheral inflammation via various modalities (see bulleted section below).

PURPOSE

In addition to facilitating target validation and clinical trial design, biomarkers aid interpretation of trial results, even if the drug fails to demonstrate desired efficacy. Therefore, MJFF aims to support development of neuroinflammation/inflammation biomarkers to garner knowledge that is critically important to the PD field – from a drug's mechanism of action and its relevance to disease biology to understanding the biological basis underlying patient heterogeneity in drug response. Within the broad scope of inflammation and immune signaling in PD, we seek funding proposals utilizing human clinical biosamples or tissues that are focused on the following modalities and subtopics:

Companion Biomarkers

- Validation of novel neuroinflammation/inflammation biomarkers for PD should be evaluated in relation to other established, non-inflammation markers that are meaningful to PD (e.g., alpha-

synuclein, GBA, LRRK2, BDNF, etc.), as well as relevant clinical measures of PD (e.g., MDS UPDRS scores)
- Analysis and sharing of clinical data from existing, or proposed studies that will be publically available for research purposes
- Advanced analytics on existing datasets

Immunophenotyping/Phenoconversion/Biochemical Assays

- Identification and validation of a neuroinflammation/peripheral inflammation endophenotype that identifies a specific, chronic inflammation "signature" for PD or that enables quantitative assessment of PD pathology/pathophysiology or disease progression
- Development of target-based, biochemical or genetic assays that could be utilized in clinical trials to select subjects or understand the impact of novel treatments on the proposed mechanism (including development of new assays/assay platforms to analyze tissues or biofluids or refinement/validation of existing assays)

Impact of aging on inflammation/neuroinflammation and relevance to PD

- As aging is a major risk factor for PD, studies aimed at elucidating how aging may impact immune signaling and thereby modify neuroinflammation/peripheral inflammation pathways in ways that may impinge on neurodegeneration in PD are of exceptional interest

The following types of biomarker studies are **not** encouraged under this initiative:

- Proposals that do not contain any preliminary data in human biofluids or tissue (novel imaging tracer development projects are a potential exception to this rule)
- Proposals that do not contain validation plans in human biosamples or tissue

BIOSAMPLE REQUESTS

Prospective studies or retrospective studies utilizing existing data in human biofluids are eligible for this initiative. Please note that studies requesting biosamples available through the collaborative Request to access Parkinson's disease related biospecimens program or the Parkinson's Progression Markers Initiative are not eligible to apply for funding through this initiative. Funding requests to support use of biosamples from these resources will be considered by the Foundation on a case by case basis through inquiry via resources@michaeljfox.org.

FUNDING AVAILABLE

MJFF intends to support multiple projects through this RFA. This funding mechanism has a budget maximum of $300,000.00 and the requested funding support should be commensurate with the stage of development and work proposed. Investigators may request support for a 1-2 year research plan and should provide a timeline with their pre-proposal. No more than 25% (for academic institutions) or 10% (for-profit organizations) of the funding may go to indirect costs. Please see the Pre-Proposal Instructions, Administrative Guidelines and our FAQ on MJFF indirect cost policy for details.

SHARING REQUIREMENTS

As MJFF is a public charity, research conducted with funds from MJFF (the "Research") must be conducted in the public interest. MJFF acknowledges that any discoveries and related regulatory approvals made by researchers through the Research are the property of those conducting and responsible for the Research and that unless otherwise agreed to by the parties, such researchers have the first opportunity to exploit the Research commercially or otherwise. Notwithstanding, each Applicant acknowledges that MJFF has the

right, after reasonable consultation, which will not be unreasonably denied, conditioned or delayed, to release a summary of findings of the Research within 90 days from the date of the grant program expiration.

In addition to the foregoing, MJFF requires that all tools, reagents, or assays (i) funded by and (ii) that result from awarded projects (collectively, the "Results") be made readily available to the community including MJFF for research purposes. You will be required to agree that you will cooperate and collaborate with MJFF and other researchers and share access to Results on fair and reasonable terms and conditions within 6 months of grant expiration. By submitting your application, you and your institution are confirming that you are not aware of any requirements that would prohibit, delay, or restrict your ability to share your Results from this initiative, including requirements of third-party collaborators or companies with which you are affiliated.

Furthermore, you agree that during the term of your Award and upon request by MJFF, you will participate in certain discussions conducted by MJFF involving other PD researchers, and you will share research information tools, and assays discussed with others involved in such discussions ("Consortium Participants"). You and all other Consortium Participants will sign the MJFF Inflammation Consortium Materials Policy, acknowledging that Participants shall treat the information, tools, and assays shared during and after such discussions as confidential information. Any information or tools you provide or receive as a result of your participation in such discussions shall be used only for the purpose of permitting Consortium Participants to advance PD scientific research or assess your Project.

ELIGIBILITY REQUIREMENTS

Applications may be submitted by:

- U.S. and non-U.S. biotechnology/pharmaceutical companies or other for-profit entities, either publicly or privately held; and
- U.S. and non-U.S. entities, 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. Please download and carefully review the program submission instructions before submitting an application.

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 www.michaeljfox.org/inflammationbiomarkers