**PARKINSON’S DISEASE THERAPEUTICS PIPELINE PROGRAM**

**Request for Applications**

**Pre-Clinical Track**

#### **pre-proposal**

*Please answer the following checkbox questions and use the template below to create your pre-proposal and upload a single PDF copy with your online submission.*

**Principal Investigator**:

**Institution/Company**:

**Project Title**:

**Academic or Industry Partner**:

(all academic applicants must have an industry partner)

I plan to request biospecimens from MJFF for use in this project. If yes, please indicate which cohort in the template below and complete the provided Biosample Request form in its entirety and upload a PDF copy with your online submission.

Please check **one** box indicating the **primary aspect of the disease** that the proposed therapeutic is intended to **directly and specifically** address, so that we can assign your pre-proposal to the correct scientific review tract. Please choose **either** a Disease symptom(s) or a Pathological underlying molecular changes category. Select a pathological underlying molecular change if that is the main, most direct target of the proposed therapeutic approach, even if the final desired outcome of the proposed therapy may well be to reduce or improve any of the motor and/or non-motor symptoms of the disease.

**Disease symptoms addressed by my therapeutic intervention**:

☐ Motor symptoms of Parkinson’s disease (PD) or as result of standard of care (gait disorder, falling, tremor, etc.)

☐ Non-motor disease symptoms (cognitive changes, apathy, depression, etc.)

Or,

**Pathological underlying molecular changes addressed by my therapeutic intervention**:

☐ Alpha-synuclein

☐ LRRK2/GBA1/Lysosomal-Endolysosomal dysfunction

☐ PARKIN/PINK1/Mitochondria-mitophagy dysfunction

☐ NLRP3/Inflammation/Neuroinflammation/Immune activation

☐ Other(s)

**TEMPLATE**

*Text should be no smaller than 11-pt font and not exceed* ***2 pages. Please carefully read the instructional text within each box and address each point in your response. Replace the instructional text with your responses in the second column of the table, maintaining the headings in the first column.*** *We encourage the use of bullet points, when appropriate, as well as the submission of schematics, images and/or graphs accompanying any unpublished preliminary data. Optional literature references as well as schematics, images and/or graphs are excluded from the 2-page limit.*

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| --- | --- |
| MEDICAL NEED | * What patient need are you seeking to address and in what target population? Please describe the patient selection biomarkers along with efficacy endpoints you plan to use in future clinical trials. |
| TARGET | * What is the molecular target, pathway, or circuitry**?** |
| TARGET FUNCTION AND EVIDENCE IN PD | * Please briefly describe **evidence in PD patients** supporting the therapeutic relevance of your target/pathway/circuitry. * If other than alpha-synuclein, LRRK2, Parkin/PINK1, GBA1, or NLRP3, please describe **how and in which pre-clinical models have the functional role of your target/pathway/circuitry been validated for PD? Please describe any relevant human testing of the target as well.** * Please share any relevant non-PD evidence for your target. |
| THERAPEUTIC APPROACH | * What is the mechanism of action of your proposed therapeutic? * **Please describe the novelty of your therapeutic approach and how your program differs from other similar approaches in the market, or in development** (especially relevant for advanced PD targets, such as alpha-synuclein, LRRK2, Parkin, PINK1, GBA1, or NLRP3, as well as other targets being actively pursued by other groups, either in preclinical programs or in the clinic). * What problems have been encountered in the past during the development of similar approaches are you trying to address with your proposed therapeutic approach? |
| EXPERIMENTAL PLAN | * What stage of drug discovery are you at (e.g. hit discovery, lead optimization, preclinical development, etc.)? * What are the specific next steps you are trying to address to further advance the proposed therapeutic approach? * When applicable, and if the proposed work includes therapeutic efficacy and/or safety/toxicity testing of lead compounds in vitro and/or in vivo, please include a few sentences regarding the chemical properties as well as pharmacokinetics profile and CNS penetrance of your leads. |
| BIOMARKERS PLAN (as relevant) | * Which biomarkers will you use to establish target/pathway engagement as well as pharmacokinetics/pharmacodynamics relationship of the proposed therapeutics? |
| INTELLECTUAL PROPERTY | * Describe any intellectual property considerations and/or restrictions (i.e. freedom-to-operate) that may impact further advancement of the proposed therapy. * For industry/academia collaborations, please describe in a few words please describe your ownership structure and roles for advancement of the therapeutic program, if successful. |
| BUSINESS PLAN & PROGRAM NEEDS | * What is the overall business and funding strategy for your company and this program? What upcoming milestones and value inflection points are critical to advancing the program? * How can MJFF support your efforts? How does this grant fit into your broader business & funding plans? What non-funding resources do you need to support your program? |
| BUDGET | * Please provide an estimate of the budget and timeframe needed to move the program forward to the next important milestone. |
| BIOSAMPLES | * If planning to request a sample cohort, please consult the [MJFF biorepository inventory catalogue](https://mjffbiobank.org/#!/biospecimens-and-data), and indicate which cohort you are planning to request samples from. You must also complete and upload a PDF copy of the attached Biosample Request form with your online submission. |