**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:

[ ]  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 indicate the main aspect of the disease that the proposed therapeutic is intended to **specifically and directly** address, differentiating between Disease symptom(s) vs. Pathological underlying molecular changes. Try to avoid selecting disease symptoms when the main purpose of the proposed therapeutic approach is to address any of the pathological underlying molecular changes of the disease, although consequently, 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:

☐ 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:

☐ 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. If desired, you may delete it to save space, but make sure headings are kept and all instructional text is addressed.*** *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use of bullet points, when appropriate, as well as the submission of schematics, images and/or graphs accompanying any unpublished preliminary data.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 counting.*

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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.
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| TARGET/MOA | * What is the molecular target, pathway, or circuitry and **how is it** **linked to PD?**
* If other than alpha-synuclein, LRRK2, Parkin/PINK1, GBA1, or NLRP3, please describe **how and in which models has the functional role of your target/pathway/circuitry been validated for PD?**
* 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, GBA, or NLRP3, as well as other targets being actively pursued by other groups, either in preclinical programs or in the clinic).
* What problems encountered in the past during the development of similar approaches are you trying to address with your proposed therapeutic approach?
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| 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.
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| BIOMARKERS PLAN (as relevant) | * Which biomarkers will you use to establish target/pathway engagement as well as pharmacokinetics/pharmacodynamics relationship of the proposed therapeutics?
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| 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 your plan to co-advance the therapeutic program, if successful.
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|  PROGRAM NEEDS | * How can MJFF support your efforts? Please provide an estimate of the budget, as well as non-funding resource needs to move the program forward to the next important milestone.
* 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.
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