**PRE-CLINICAL THERAPEUTICS PIPELINE PROGRAM**

**Request for Applications**

#### **Letter of intent**

*Please answer the following checkbox questions and use the template below to create your letter of intent and upload a single PDF copy with your online submission. Text should be no smaller than 11-pt font and not exceed* ***2 pages. Checkboxes, as well as optional literature references, are excluded from the 2-page counting.***

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 below template. You will be asked to provide more documentation on this request if invited to submit a full proposal)

The proposed therapeutic is intended to address the following aspects of the disease (please indicate only direct targets and avoid selecting pharmacodynamic readouts):

Specific disease symptoms:

☐ Motor symptoms of PD or as result of standard of care (gait disorder, falling, tremor, dyskinesia...).

☐ Non-motor disease symptoms (cognitive changes, constipation, fatigue, depression...)

Underlying disease progression:

☐ Alpha-synuclein pathological processes

☐ LRRK2/GBA1/Lysosomal-Endolysosomal dysfunction

☐ PARKIN/PINK1/Mitochondria-mitophagy dysfunction

☐ Inflammation/Neuroinflammation

☐ Other(s)

**TEMPLATE**

*Text should be no smaller than 11-pt font and not exceed* ***2 pages.*** *You may delete the instructional text in each box of the template to save space.*

|  |  |
| --- | --- |
| MEDICAL NEED | What need are you seeking to address in what target population? Have patient selection biomarkers to stratify patients in future clinical trials already been developed? If so, which are those?  |
| TARGET/PATHWAY/CIRCUITRY | What molecular target, pathway, or circuitry have you selected? How is it linked to PD? How and in which models has the functional role of your target/pathway/circuitry been validated in PD? This is not necessary for well validated targets such as alpha-synuclein, LRRK2, GBA1, or PARKIN/PINK1.  |
| EXPERIMENTAL PLAN | What stage of drug discovery are you at (e.g. hit discovery, lead optimization, preclinical development, etc.)? What are the key gaps you are trying to address? |
| BIOMARKERS PLAN (as relevant) | Which biomarkers will you use to establish target engagement/modulation of the therapeutics? How will you establish PK/PD relationship and blood-brain-barrier penetration if your therapeutic is CNS-penetrant?  |
| 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 |
|  PROGRAM NEEDS | How can MJFF support your efforts? Please provide a high-level description of resource needs to move the program forward to the next important milestone. If requesting financial support, you can include an estimate of the budget that would be needed to complete the study.Is there specific access to expertise, advice, or reagents that MJFF can help facilitate? |