



MULTI-MODAL THERAPIES FOR PARKINSON'S DISEASE

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The Michael J. Fox Foundation for Parkinson's Research

MJFF IS THE WORLD'S LARGEST NONPROFIT FUNDER OF PD RESEARCH

Our Mission

To accelerate the development of improved therapies, and ultimately a cure, for people living with Parkinson's disease today.

Vital Stats

- » Founded in **2000** by actor Michael J. Fox
- » Public charity
- » Nearly **70,000** donors in 2015
(individuals, corporations, nonprofits)
- » No chapters: team of **100** based in NYC
- » **3,300** grassroots fundraisers reaching
150,000 supporters worldwide in 2015
- » More than **\$600 million** in research programs
funded to date
- » **\$87.8 million** in research programs funded in 2015
- » Nearly **2,100** projects funded to date
- » **550** active grants in current portfolio
- » **33%** of funded projects are led by researchers
outside the United States
- » Fund academics, biotechs and pharma



“COMBINATION THERAPIES” ARE BEING USED TO TREAT PD

Combination Therapies to Enhance Delivery or Response of a Drug?

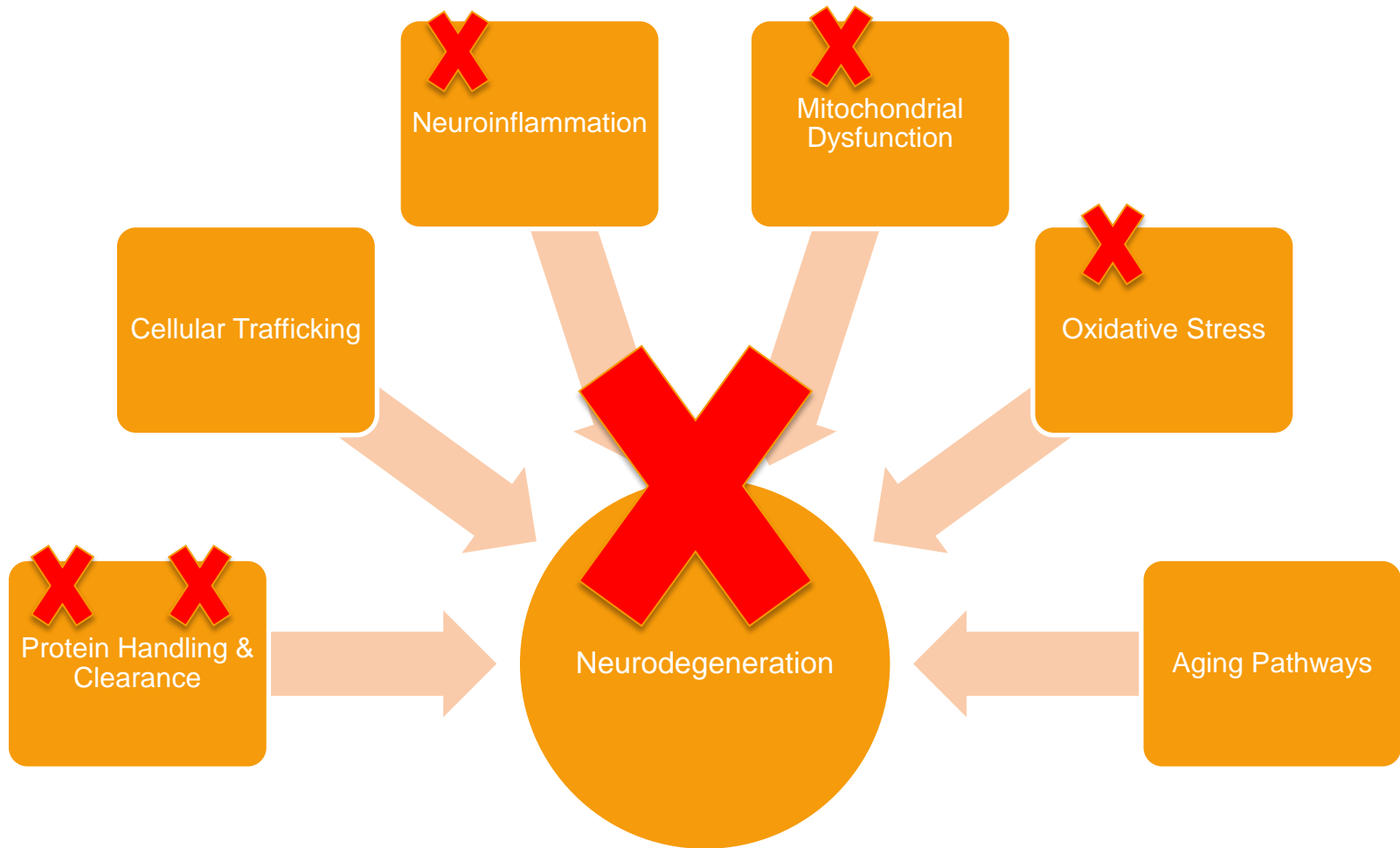
- Dopamine replacement & restoration (Sinemet, Stalevo, Duopa, etc.)
- Surgical (DBS) in combination with medication
- Gene/Protein delivery of investigational neuroprotective therapeutics via device, FUS or engineered cellular therapy

Combination Therapies to Target Multiple Disease Symptoms & Mechanisms?

- Motor/Non-motor symptomatic “combination” therapy
- Combination therapies targeting multiple disease mechanisms are currently lacking



CAN COMBINATION THERAPY TARGET PD DISEASE CAUSE AND MECHANISM?



COMBINATION THERAPIES FOR PD DISEASE MODIFICATION

Key Challenges

- » Insufficient understanding of PD mechanism(s) makes it difficult to build rationale argument for any particular combination therapy
- » A nearly infinite number of permutations of combination therapies but limited resources (funding, infrastructure, patients)
- » Regulatory and Payer challenges (although these are not PD specific)

Some Recommendations

- » Continue push for greater understanding of PD biology in the best model system: the patient!
- » Greater integration and sharing of data to develop clearer picture of shared or distinct disease biology
- » Establish clearer biomarkers and outcomes linked to disease biology as well as patient-reported outcomes (ASYN Imaging, web/mobile, etc.)
- » Use of novel approaches (e.g., Platform trials?) in the PD (or related neurodegenerative?) space

