



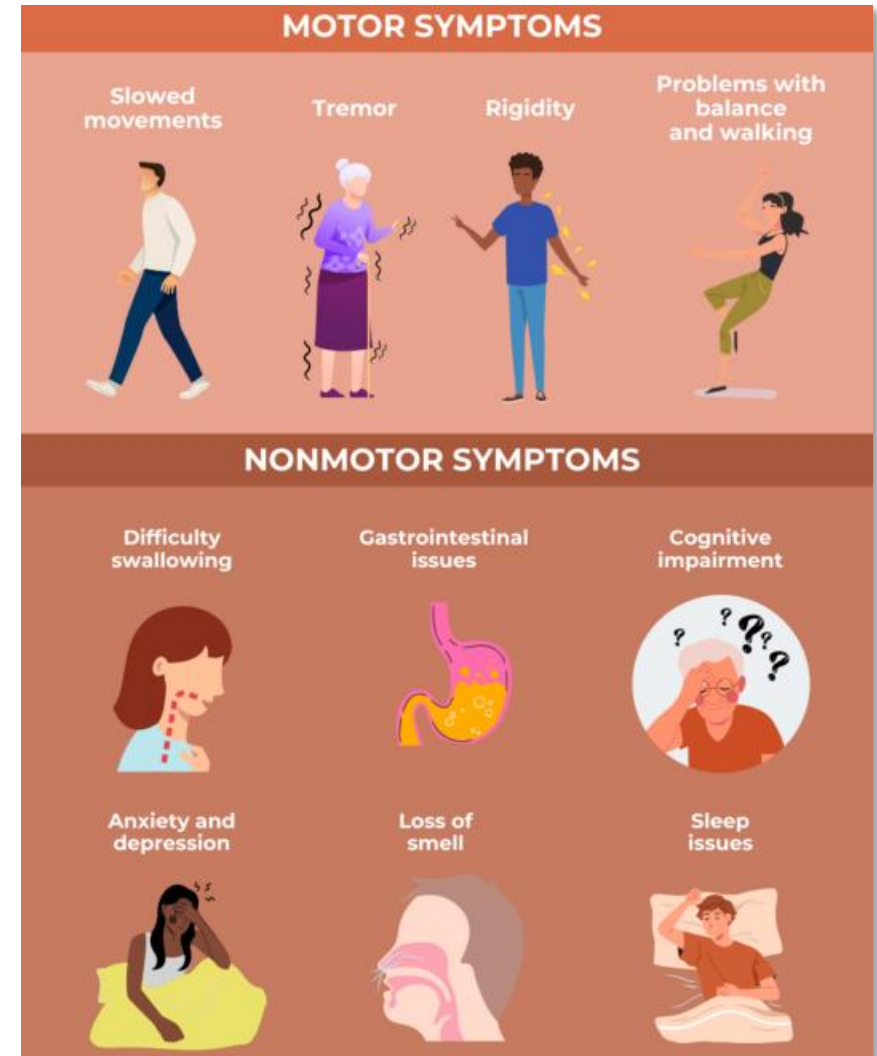
MJFF
TRANSLATIONAL RESEARCH

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WHAT IS PARKINSON'S DISEASE?

- **Second most common** neurodegenerative disease
- Majority of cases do not have a known cause
 - **10% of cases have known genetic link**
 - Risk factors include age, head injury, environmental exposures (pesticides, heavy metals, toxins).
- Classified as a movement disorder
 - Diagnosis based on motor symptoms
 - **Non-motor symptoms can occur decades before diagnosis and progress with disease duration**
- Treatment strategies are solely **symptom management**
 - Motor: dopamine replacement, dopamine agonists, deep brain stimulation
 - Non-Motor: traditional treatment strategy for symptoms in other indications



THE STATE OF PARKINSON'S DISEASE RESEARCH



- + Genetics and related biology have exponentially expanded disease understanding, but *we need more data to support new biology-informed and globally diverse disease definitions*
- + Growing list of treatments address symptoms, but *we need more confident translation of biology and sustained investment in high-risk/high-reward disease-slowing treatments*
- + Clinical trial pipeline is robust, but we *need more informative measurement tools and efficient and inclusive study designs to speed trial throughput*
- + PD healthcare is improving, but we *need to empower the broader stakeholder community with information and clarity on critical patient needs and options*



THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH

- + Founded in 2000 by actor Michael J. Fox
- + Global strategic funder and facilitator: more than \$2 billion deployed to a mission-driven strategic research agenda centered on advancing PD research and drug development
- + Accelerating cures by connecting community: people with Parkinson's, care providers, researchers, industry, investors, regulators, payers, policy-makers, and other strategic partners



THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

Learn more at
www.michaeljfox.org

Here. Until Parkinson's Isn't.



FROM VISION TO MISSION

MJFF seeks to deliver unique value through a mission centered on a strategic research agenda

Our Vision

A World Without Parkinson's Disease

- + Early detection of people at risk before onset of symptoms
- + Available treatments prevent onset, progression and impact of symptoms to maintain and restore function
- + Healthcare systems deliver quality and accessible care to all who need it

Our Mission

To Go Out of Business

"The Michael J. Fox Foundation is dedicated **to finding a cure** for Parkinson's disease through an aggressively funded [strategic] research agenda and to ensuring the development of improved therapies for those living with Parkinson's today."



THE MJFF STRATEGIC RESEARCH AGENDA



MJFF is focused on moving projects through the therapeutic development pipeline to ultimately de-risk the PD field for larger funders by:

- Launching strategic funding programs for key questions
- Addressing field-wide challenges in precompetitive initiatives
- Funding high-risk, high-reward projects
- Providing non-dilutive funding

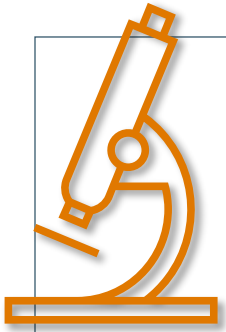
Early in our history, we realized the quality and availability of tools were make-or-break for our success:

- Research grants experienced extensive delays due to inability to access the right tools
- Outcomes of grants were inconclusive or uninterpretable due to poor quality tools/models
- Successful projects couldn't be replicated or extended as tools were not widely accessible.



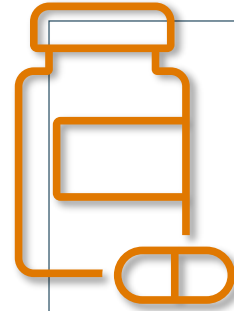
Framing Our Strategic Research Agenda

High-level outcomes emphasize where we want to go to move us closer to cures



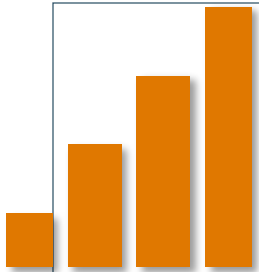
Clearer Disease Definition

- More biology-centered
- More globally relevant



Better Treatments

- More confident biology translation
- More robust and diverse pipeline



Faster Clinical Trials

- More meaningful disease measures
- More efficient and inclusive designs



Quality Healthcare for All

- Pro-PD policies and practices
- Educated providers/community



Turning Agenda into Action

Progress needs seen through patient-centered perspective should shape our program priorities

Outcome	Prevent Disease	Slow Progression	Restore Function	Cross-Cutting Principles
Clearer Definition	Define risk factors and earliest biological features	Define molecular subtypes linked to clinical impact	Define biological drivers of most meaningful symptoms	+ Establish open resources to accelerate research
Better Treatments	Develop treatments against earliest biological features	Develop treatments matched to biology	Develop treatments addressing most meaningful symptoms	+ Leverage key partnerships to amplify impact
Faster Clinical Trials	Establish measures for early detection and prevention	Establish sensitive measures of biology and progression	Establish sensitive measures of patient-relevant function	+ Connect an engaged and diverse community to guide and drive meaningful change
Healthcare for All	Reduce environmental contributors to risk	Address care access and treatment adoption barriers		

Mapping Our Programs

Our initiatives ideally seek to address one or more barriers to progress

Outcome	Prevent Disease	Slow Progression	Restore Function	Cross-Cutting Principles
Clearer Definition	ASAP Global Parkinson’s Genetics Program (GP2) ASAP Collaborative Research Network (CRN) Parkinson’s Progression Markers Initiative (PPMI) – Natural History Study MJFF Subtype Discovery Programs			Tools and Resource Programs Stakeholder Alliances Community Outreach and Education Programs
Better Treatments	MJFF Target Advancement/”Targets to Therapies (T2T)” Program MJFF Parkinson’s Therapeutic Pipeline (TPP)			
Faster Clinical Trials	MJFF Measurement Initiatives (Biomarkers, Imaging, Clinical Endpoints) PPMI Measurement Validation Programs			
	Path to Prevention (P2P) Platform Trial	Inclusive Recruitment and Retention Programs		
Healthcare for All	Pesticide Policy and Advocacy Action Plan	State Registry Programs/CDC Surveillance Program Physician Training and Education Programs Market Access and Adoption Intelligence Programs		
	National Plan to End Parkinson’s Disease			

How We Act and Operate

We balance and leverage multiple approaches and mechanisms to address our programmatic needs

Important Ways We Offer Support

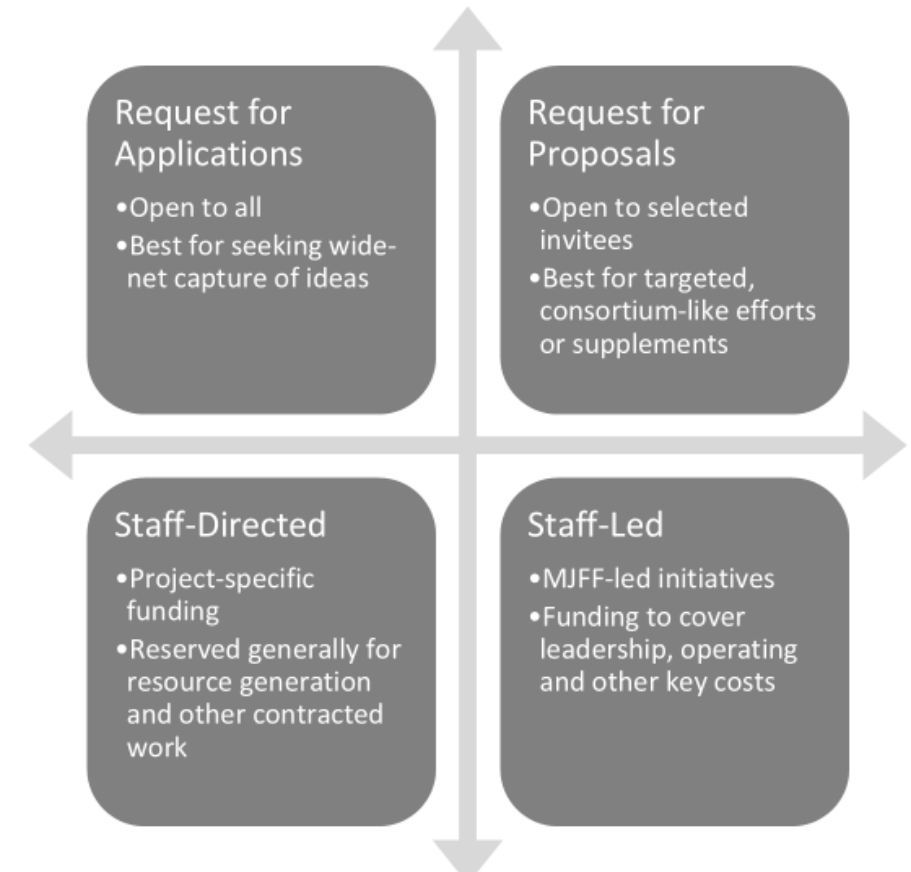
Funding

- Investigator grant funding around key topics and areas of interest
- Initiative and Partner Program implementation and funding

Facilitation

- Convening and Strategic Consensus-Building
- Research Resource Development and Distribution
- Education and Advocacy

Important Mechanisms We Use



Partnerships are Essential to Our Impact

Consortia and public-private partnerships help identify field challenges and jointly overcome hurdles



PARKINSON'S DISEASE RESEARCH
TOOLS CONSORTIUM
THE MICHAEL J. FOX FOUNDATION



Parkinson's
Progression
Markers
Initiative

The Michael J. Fox Foundation presents

Parkinson's
IQ+YOU

Education for Empowerment



Parkinson's Disease
Education Consortium

We support implementation of ASAP's strategic research roadmap to amplify impact



Aligning Science
Across Parkinson's



Collaborative
Research Network



Global Parkinson's
Genetics Program



THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

Thank you!

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MJFF ACCESS DATA PROGRAM

- » MJFF provides access to data from **10 studies through 5 research gateways**, some of which also include patient biosamples.
- » Between the various resources, **8 different types of data** are available.



Clinical



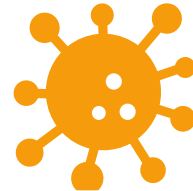
Imaging



Genetic



Transcriptomic



Proteomic



Biological



Wearable Device



Patient Reported Outcomes

- » To date, we've had **>15 million downloads** of these datasets by researchers.

Information on data sets and links to data gateways can be found at <https://www.michaeljfox.org/data-resources>. Information on our Parkinson's Progression Marker's Initiative (PPMI) is at <https://www.ppmi-info.org/access-data-specimens/download-data/>.

