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# PARTNERING TO DEVELOP BETTER PARKINSON'S TREATMENTS

Resources to accelerate your Parkinson's research

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# WORKING WITH US

Patients are waiting for new and improved treatments for Parkinson's. Fortunately, people like you are working at every stage of research to accelerate knowledge turns and rapidly advance drug development toward better outcomes for people living with Parkinson's disease (PD). The past two decades have brought substantial progress in the science of Parkinson's including: (1) a greater understanding of Parkinson's disease biology; (2) the translation of biology to therapies being tested in the clinic; and (3) an open, collaborative research environment supporting continued discovery and innovation. Now more than ever, there is an opportunity to build on this groundswell of collective knowledge and resources available to researchers to advance science and address the unmet needs of the Parkinson's patient community. While we have made progress, much remains to be discovered — and the field's continued growth will come from dedicated researchers like you. The Michael J. Fox Foundation (MJFF) is here to help.

Working at the global nexus of Parkinson's research, MJFF is uniquely positioned to cultivate and maintain a field-wide perspective on new learnings and emerging challenges. The ever-evolving research landscape shapes the next steps in the journey to attain our singular goal: new and improved treatments, up to and including a cure, for Parkinson's. At any given moment, our aim is to prioritize the field's most important activities, articulate their importance, gain stakeholder buy-in, and provide funding and technical assistance to push them forward. Today, MJFF is a major and irreplaceable funder of Parkinson's science with sound rationale and clear patient relevance. We have funded over \$1 billion in research since inception and strive to fund at least \$100 million in research programs annually.

But MJFF is more than a funder — we strive to be a partner in the success of your projects. We also offer a robust catalog of tools and resources to advance your work, and we aim to provide opportunities for productive collaboration with key stakeholders across the Parkinson's community. MJFF is committed to providing support and resources to those invested in Parkinson's research — across academia, industry, government, health tech, venture capital, and more — we are all part of an ecosystem making important discoveries and advancing novel Parkinson's therapies. In the 20+ years since MJFF was "As a basic scientist and representative of the biomedical community, I greatly appreciate the efforts of The Michael J. Fox Foundation to create programs in support of patients and the research community who share the goal of controlling this terrible disease."

Randy Schekman, PhD, Nobel Prize
 Winner and ASAP Scientific Director

Stay up to date on funding opportunities, resources and news to accelerate research and patient care by by joining our e-mail list.



established, the Foundation has evolved to work closely with the research community in a variety of ways, all with the goal of supporting and accelerating Parkinson's research.

This document is meant to serve as a roadmap to guide you to the available resources and opportunities for collaboration that will meet the unique needs at every stage of your research and drug development efforts. You'll find an overview of our available laboratory tools, data and biospecimens, and recruitment and retention resources — our aim is for this resource to make it easy to find what you need, when you need it, so that you can spend more time focusing on your research. Members of the MJFF team are available to you for discussion on these resources and to explore further ideas for collaboration. To connect with a member of the MJFF team, please e-mail **researchpartnerships@michaeljfox.org**.

We thank you for your continued commitment to Parkinson's research and to helping MJFF achieve our mission. We are dedicated to helping your research be equally successful.

### **MJFF's Research Network**

#### **MJFF's In-house Research Staff:**

With approximately 15 PhDs providing scientific guidance and 40 staff working across grants management, clinical operations, public policy, patient engagement, and partnership efforts, The Foundation is positioned to maintain a field-wide perspective on Parkinson's research and execute a strategic research vision through funding and other investments.

A Diverse Network of Key Opinion Leaders: Researchers on MJFF's **Executive Scientific** Advisory Board provide guidance and feedback to MJFF staff, and a broader global network of experts review grant proposals, assess project outcomes and share input on MJFF initiatives. In 2020, over 250 advisors helped drive progress toward MJFF research goals. The MJFF Patient **Council**, a group of over 30 people living with Parkinson's, work closely with the Foundation to help ensure MJFF strategy and initiatives are informed by the patient voice. MJFF's diverse network of experts means the Foundation can be a resource for you in sharing expertise across a wide range of areas in Parkinson's research, including biology, biomarkers, outcome measures, data science, clinical development, regulatory science, and patient perspective.

### Active Collaborations with Other Multistakeholder Research Initiatives:

MJFF participates in key pan-organization initiatives in the Parkinson's ecosystem across research, patient engagement, and regulatory science — meaning MJFF stays up to date on the latest in these initiatives and can help point you to the right organizations based on your needs. These initiatives include the Critical Path for Parkinson's initiative, Accelerating Medicines Partnership Parkinson's disease (AMP-PD), and close interaction with NIH. MJFF is also the implementation partner for the Aligning Science Across Parkinson's (ASAP) initiative, which fosters collaboration and resources to better understand the underlying causes of Parkinson's disease.





# ACCESS RESOURCES TO ACCELERATE YOUR PARKINSON'S RESEARCH

We want to make Parkinson's research easier. In addition to funding millions of dollars in research, MJFF spearheads our own key initiatives to help advance understanding and treatment of Parkinson's disease. Our landmark programs and resources are changing the landscape of Parkinson's research, hopefully enabling you to more easily and successfully advance toward your goals. You can access a robust range of resources through MJFF including critical funds, well-characterized laboratory tools, clinical data and biospecimen from Parkinson's cohorts, and detailed landscape reports.

# **Funding Opportunities**

MJFF provides non-dilutive funding to academic and industry scientists, small biotechs, big pharma and allied care professionals. Our portfolio spans basic, translational and clinical research. In addition to grant support, awardees benefit from working with MJFF internal research staff and its broad network of scientific advisors. Regardless of funding decision, MJFF scientists provide expert feedback on all reviewed grant proposals to support future submissions to MJFF or other funders. MJFF's primary mechanism for soliciting ideas in Parkinson's research is through regular, open request for application (RFA) programs.

Current core RFA programs include:

- Target Advancement
  build robust evidence to rationalize biological pathways and targets for further translation into Parkinson's
  biomarkers or therapies
- + Therapeutic Pipeline develop and test novel treatments
- + Outcome Measures identify and characterize biomarker tools and clinical outcome measures to assist in trial design and interpretation of results
- + Edmond J. Safra Fellowship in Movement Disorders annual funding to academic centers to train a new movement disorder clinician-researcher over a two-year period

**For more information**: View our open **funding programs**. Reach out to **grants@michaeljfox.org** if you have questions about applying or would like to learn more.



### Data Resources and Biospecimens

We believe data sharing is critical to research progress. MJFF collaborates with the research community to collect and distribute a multitude of data (including, but not limited to, clinical, ePRO, imaging, 'omics and sensor data) as well as biosamples from well-characterized Parkinson's cohorts. Through centralized repositories, you can more quickly find and access resources to advance discovery and validation research.

For more information: Visit the MJFF Data Resources Page and the MJFF Biospecimen

**Overview Page** for a snapshot of Parkinson's cohorts and available biospecimens. You can also directly search for specimens and apply for access on the MJFF **Biorepository Website**. The large majority of MJFF biospecimens are available at no cost to researchers.

If you have questions about any of our available data or biospecimen resources, please reach out to **resources@michaeljfox.org**.

### **Research Tools**

To save researchers time and resources, MJFF makes well-validated laboratory tools—such as antibodies, research-grade assays, and animal models—available to the scientific community at low cost, with rapid delivery. Our tools catalog is searchable by tool type and by gene/ protein type (e.g., alpha synuclein, PINK1) to make it easy to find what you need. Additionally, MJFF scientific staff can answer questions and provide guidance on the many different pre-clinical models used in Parkinson's disease research to help research teams design their studies.

For more information: Search the catalog. You can get answers to questions about available resources or suggest new tools for development by emailing tools@michaeljfox.org.

## MJFF and Digital Health

MJFF is committed to supporting advances in digital health including medical devices that have the potential to help the Parkinson's community.

If you are working in the digital health space and interested in opportunities to collaborate with MJFF, please reach out to **researchpartnerships@michaeljfox.org** 

### Parkinson's Disease Landscape Reports

MJFF actively tracks key developments in the Parkinson's landscape, from advancements in preliminary pre development research to drugs entering late-stage clinical trials. We summarize the key trends, identify gaps, and highlight areas of focus moving forward- not just for the Foundation itself, but for the broader PD Research community.

- + Clinical Pipeline Report provides a snapshot of drugs in clinical development, along with high-level perspectives on priority targets and modalities. This report serves as an excellent primer for those newer to working in Parkinson's. To request access to the Clincial Pipeline Report, please email researchpartnerships@michaeljfox.org
- + Economic Burden and Future Impact of Parkinson's Report — details the economic impact of Parkinson's disease in the U.S., including prevalence and medical and non-medical costs associated with PD; data in this report may be a useful tool for HEOR teams to assess the potential economic benefit of novel Parkinson's therapies. Download the 2019 Economic Burden of Parkinson's Report.
- HJFF Target Report provides a brief overview of targets of interest from MJFF's research portfolio. All information included in this report is publicly available and is intended to be used as a resource for researchers and investors evaluating opportunities and/ or seeking new projects. Download the January 2022 Target Report.



### **Research Roadmap**

MJFF takes a comprehensive approach to Parkinson's research. By funding basic research through clinical development of treatments, MJFF contributes to breakthroughs in understanding the causes of Parkinson's and translating those findings to new tests and therapies.

Our strategy comprises programs to:

- + **Define** the causes of Parkinson's, its progression, and the factors that account for the variability of the disease
- Measure biological and clinical markers and features to diagnose Parkinson's, track its progression, and assess the effectiveness of treatments
- + **Treat** the underlying pathology to slow, stop or prevent Parkinson's progression, and develop new therapies to alleviate symptoms
- Define Enable Measure
- + **Enable** rapid knowledge turns by providing tools and resources, from assays to data to trial support, that the field needs

# Case Study: MJFF as a Funder of Early-Stage Therapies

Many projects with sound scientific rationale and the potential to make a difference in patient lives lack the financial resources to properly vet their approach. Many MJFF-funded companies have attracted collaborations and acquisitions that sped their pipeline. The following are two of dozens of projects that have received follow-on funding after MJFF de-risking grants:

Biotech company, **Neuropore**, developed a small molecule targeting alpha-synuclein for disease modification with close to \$750,000 in pre-clinical funding from MJFF. **Neuropore** entered a collaboration with **UCB** in 2015 with a potential payment of up to \$460 million. In January 2020, the company announced it had successfully completed a Phase I clinical trial and was planning its next study.

**Inbrija (inhaled levodopa)** for "OFF" episodes is the first MJFF-funded therapy approved by the U.S. Food and Drug Administration. MJFF partially funded Phase I and II trials of Inbrija by biotechnology company **Civitas Therapeutics** in 2011 and 2013 with two grants totaling \$1.3 million. **Acorda Therapeutics, Inc.** acquired Civitas in 2014 and continued the development of **Inbrija** toward approval in 2018. (Sunovion's Kynmobi (sublingual apomorphine) approval in 2020 for the same indication marked the second of a therapy supported with MJFF funds.)





# COLLABORATE TO SPEED INSIGHTS

Collaboration is crucial to accelerating progress in the field of Parkinson's research. To enable researchers to move their programs forward most efficiently, the Foundation organizes a range of initiatives to bring together experts to tackle the field's most pressing questions. Our consortia and meetings are open to academia, industry groups and CROs and facilitate resource and data sharing through pooling of intellectual and financial capital.

## Parkinson's Progression Markers Initiative (PPMI)

PPMI is our landmark observational study to better define and measure Parkinson's disease to speed therapeutic development. PPMI makes its dataset and biorepository — the most robust in Parkinson's to date available to researchers to accelerate breakthroughs.

Launched in 2010, PPMI has gathered longitudinal data from more than 1,400 individuals at 33 clinical sites in 11 countries. The study is growing exponentially to enroll a larger group of individuals — de novo Parkinson's, control volunteers and at-risk populations — who can contribute to a more detailed look into the way the disease develops and changes over time.

PPMI is sponsored by MJFF with support from more than 40 industry, non-profit and private partners.

**Benefits of Membership:** PPMI partners provide feedback on study parameters through the Partner Scientific Advisory Board. Through close interaction with the study, partners are positioned to shape study direction and gain access to real-time learnings relevant to clinical development through regular teleconference and working group meetings.

**Commitment:** Industry partners are contributing to PPMI through financial and in-kind donations.

### PPMI by the Numbers

22,000+ study participants enrolled

100,000+ participant recruitment goal

8 million+

downloads of PPMI data by researchers

> 40+ industry partners

20+ interventional trials with study design informed by PPMI data

Learn more about PPMI. MJFF is eager to partner with industry organizations to make PPMI a success.



## Global Parkinson's Genetics Program (GP2)

This worldwide program aims to redefine the genetic architecture of Parkinson's disease and make this knowledge globally relevant. GP2 will genotype over 150,000 individuals with a special focus on diverse ancestry groups and rare familial forms of PD. This research is made possible through collaboration with member institutions, scientists, and researchers from around the world who submit cohorts of volunteer research participants; GP2 is funded by the Aligning Science Across Parkinson's (ASAP) initiative, and MJFF serves as the implementation partner for the program.

# View cohorts contributing to the study and learn how to access GP2 data.

**Commitment:** There is no cost to contribute cohorts to GP2 or to access its data.

### Parkinson's Research Tools Consortium

MJFF and industry collaborators work to identify, develop, characterize and validate new pre-clinical tools that address current scientific gaps and accelerate experiments. Through regular teleconferences, Consortium members discuss fieldwide needs and priorities in addition to data on individual tools in development. With the Consortium's guidance, MJFF works with CROs to develop, characterize and distribute new assets to the scientific community with quick delivery and at low cost. Join the Foundation and Consortium members to develop accessible and well-characterized laboratory tools to investigate the disease and impact of novel therapeutics.

**Benefits of Membership:** Recommend and provide strategic direction for the development of new research tools while saving time and money by engaging in regular strategic and technical discussions with MJFF and other experts. **Commitment:** Financial commitment required.

For more information, please contact resources@michaeljfox.org.

### MJFF's Parkinson's Disease Research Exchange Consortium (PDRx)

MJFF has merged our separate consortia calls into a single series. The PDRx will serve as a global network of scientists from academic labs, pharma/biotech companies and CROs and will include investigators focusing on preclinical and/or clinical efforts.

PDRx calls occur every Wednesday at 11 a.m. US ET with the following tracks:

- + Parkin/PINK1/Mitochondrial Pathways (monthly)
- + LRRK2/Endolysosomal Pathway (monthly)
- + GBA/Lipid Pathways (bi-monthly)
- + Synuclein/Protein Aggregation Pathways (monthly)
- + Inflammation and Immune Regulation (quarterly)

**Benefits of Membership:** Early knowledge of MJFF funding opportunities and information on MJFFgenerated pre-clinical tools as well as the abilities to help shape MJFF strategy and to learn of new and complementary efforts in the biology space with potential for collaboration opportunities.

**Commitment:** Complimentary membership for MJFF grantees and other researchers willing to share pre-publication data in confidential setting.

For more information or to join PDRx, please contact researchexchange@michaeljfox.org.



### Workshops, Summits and Conferences

The Foundation facilitates standalone annual meetings and workshops to bring together researchers from industry and academia to help accelerate therapeutic development. Our signature event is the Parkinson's Disease Therapeutics Conference, an annual scientific conference and the only conference in the world focused exclusively on Parkinson's drug development. Our summits and workshops focus on specific areas of research such as priority therapeutic targets, neuroimaging and clinical trial design and bring together scientific experts across disciplines to address field-wide challenges.



**Commitment:** Ticket purchase and registration is required for the PD Therapeutics Conference. For added exposure and visibility to a highly engaged, PD- focused audience, **sponsorship opportunities for the conference are also available**. For more details, email **researchpartnerships@michaeljfox.org**.

## Webinars for Researchers

MJFF offers free webinars on topics of interest to researchers, clinicians and industry professionals including through the Parkinson's Disease Therapeutics Webinars series that complements our in-person annual conference. Previous webinars — all available anytime on-demand — have covered topics such as precision medicine for Parkinson's, molecular signatures of disease and novel strategies to capture patient outcomes.

Commitment: For more information, contact researchpartnerships@michaeljfox.org.

Learn more about webinars.

Researcher and Clinician Email List Stay updated with the latest news from MJFF including funding opportunities, new resources, and events by joining our researcher and clinician email list.





# USE PATIENT VOICES TO TRANSFORM YOUR PARKINSON'S RESEARCH

MJFF takes a patient-centered approach to our work, and we know that Parkinson's science is better when it includes the patient voice. We sit at the nexus of the Parkinson's community, helping build critical alignment between researchers and people affected by the disease. Over the past two decades, the Foundation has cultivated a broad network of patient advocates and advisors through grassroots connections and strategic community and individual patient partnerships including the Foundation's Patient Council. We rely on these Parkinson's experience experts to provide key perspectives that drive Foundation strategy and initiatives. MJFF can be a resource as you strive to integrate the patient perspective into your work — no matter what stage of development you are in. In addition to accessing the resources noted below, you can reach out to **researchpartnerships@michaeljfox.org** to explore other ways to incorporate the patient voice in your research.

## Patient-focused Research Support

In recent years, regulators have underscored that the patient voice is essential in medicines regulation. Patientfocused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs and priorities are captured and meaningfully incorporated into drug development and evaluation. MJFF is eager to be a resource to Parkinson's researchers looking to incorporate principles of PFDD into their work across stages of development.

Email researchpartnerships@michaeljfox.org to discuss the activities below or to explore other ways to incorporate the patient voice in your research.

### **VOICE OF THE PATIENT**

- + MJFF can facilitate connections with members of the Parkinson's community to help you integrate their perspectives into your research. We can assist in, for example, identifying individuals to provide feedback on a trial's schedule of activities or to participate in an advisory board about their lived experience with Parkinson's.
- + The Fox Insight study collects patient-reported data using validated and novel instruments from people with and without Parkinson's. With over 53,000 participants contributing data, Fox Insight study data can help validate patient perspectives in a large cohort. Data are open access, and data requests can be submitted through this link FoxDen.



### **PATIENT-CENTERED ENDPOINTS**

+ MJFF is funding the development of novel, patient-centered endpoints for use in PD trials. We envision a future with more sensitive trial endpoints that align the interests of clinical trialists, regulators, payers and, most importantly, patients. Reach out to our team to learn more about ongoing, MJFF-funded projects and collaboration opportunities related to patient-centered endpoints.

#### PATIENT PREFERENCE RESEARCH

 HJFF is investing in the generation of quantifiable patient preference data as a resource for groups developing therapeutic interventions for PD. As this work is completed, findings will be published and freely available for members of the research community to access.

### Including People with Parkinson's Disease in Clinical Study Design and Execution: A Call to Action



We know that Parkinson's researchers want to engage patients. The question MJFF gets most often get is how to make this engagement meaningful. A 2022 publication — co-authored by individuals living with Parkinson's including members of MJFF's Patient Council — provides recommendations on how to successfully include members of the PD community in research. **Read the full article**.

### **Recruitment and Retention Resources**

### **FOX TRIAL FINDER**

Our online matching tool connects potential volunteers with clinical study teams. The lay-friendly format allows sponsors and research teams to customize study descriptions to make your trial more accessible to participants, increasing awareness and helping you enroll faster.

For more information: Learn more about Fox Trial Finder and complete this form to connect your active clinical trial and track volunteer referrals through our site.

#### **TRIAL RESOURCE PACK**

Leveraging experience gained through our network of leading clinical trialists and 20+ years working handin-hand with the Parkinson's community, MJFF has developed a suite of guides that equip sponsors and clinical trial teams with data-driven best practices and resources to recruit and retain study participants.

For more information: Download the trial resource guides.



## Opportunities for Community Engagement and Education

MJFF partners with industry across several programs to deliver trusted educational resources and to foster connections with the Parkinson's community. These initiatives include the Parkinson's Disease Education Consortium, the MJFF Buddy Network, Community Athletic and Special Events, and more.

For more information: learn more about how your organization can get involved in these important initiatives.

### Diversity, Equity, and Inclusion in Parkinson's Research



MJFF is committed to normalizing diversity, equity and inclusion (DEI) in Parkinson's research. Some of our latest investments in this area include:

- + Launch of a \$2M funding program to support Parkinson's studies in historically underrepresented populations
- + Top-down commitment and specific site training and resources to expand diversity in our flagship study, the Parkinson's Progression Markers Initiative (PPMI)
- + Pilot program to connect MJFF applicants and grantees with experts in inclusive trial practices to refine recruitment and retention strategies
- + Funding for projects to increase knowledge of Parkinson's genetics in populations in Africa, East Asia, India and Latin America
- + In partnership with the ASAP initiative, financial and intellectual support for the Global Parkinson's Genetics Program (GP2) to generate Parkinson's genotypic data from 150,000 individuals around the world, including from many historically underrepresented communities
- + Evaluation of funding applicant demographics as a first step in ensuring inclusive grant-making processes

As we grow our portfolio of initiatives and directives that aim to welcome and engage underrepresented populations in research and increase access to care for all, we want to hear from you on your DEI priorities. Please reach out to **researchpartnerships@michaeljfox.org** to explore ways to work together.

"There are no borders when you are living with a neurological disease, but there are barriers we must overcome. It is a priority that we reach underrepresented populations who are so critical to filling knowledge gaps about Parkinson's disease."

— Debi Brooks, CEO and Co-Founder, The Michael J. Fox Foundation





## **Policy and Advocacy**

MJFF leads the Unified Parkinson's Advocacy Council comprising representatives from state, regional and national Parkinson's organizations. The council informs our Foundation's strategy to advocate before the U.S. Congress and state legislatures as well as executive agencies to further legislative and regulatory goals on behalf of the Parkinson's community.

Ongoing policy priorities include efforts to (i) increase investments in Parkinson's research; (ii) ensure research freedom; (iii) address issues that may impact drug and device development and approvals; (iv) ensure access to quality, affordable, accessible health care; (v) collect incidence data such as through enactment of state-based Parkinson's registries; and (vi) limit environmental triggers of Parkinson's.

The voice of the research community is of significant value in our advocacy efforts. Learn more and get involved at **michaeljfox.org/advocacy** or email **policy@michaeljfox.org**.



"The most credible voice on Parkinson's research in the world."

# The New York Times



**3,200**+

"The thing about the Fox Foundation that to me is truly unique is that it's the combination of people with Parkinson's, academic research scientists, people in industry and then scientists within the Foundation, all working together as a team. That is really an efficient way to move things forward."

— Carlie Tanner, MD, PhD University of California, San Francisco; Co-Principal Investigator, PPMI

# CONNECT WITH US

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