2020 Parkinson's Disease Therapeutics Webinars

Overview & Resources



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Welcome

We hope you enjoyed our first Parkinson's Disease Therapeutics Webinars series. The Michael J. Fox Foundation (MJFF) appreciates the value of collaboration and bringing the Parkinson's research community together to share ideas and present new findings.

Often, we do that at meetings and conferences. COVID-19 required us to take a different approach this year.

MJFF created the free series of Parkinson's Disease Therapeutics Webinars to provide a forum for us to discuss the latest research and hot topics in our field.

Though the pandemic has impacted our work, Parkinson's research is moving forward. In 2020, the U.S. Food and Drug Administration approved two new drugs and one medical device for Parkinson's. And the pipeline of therapies in development is stronger and more diverse than ever. This momentum is being buoyed by progress in biomarkers. MJFF is supporting the development of an alpha-synuclein PET tracer, and other outcome measures are on the horizon.

Parkinson's research has never been easy, and this year brought us an unexpected set of challenges. But it also provided an opportunity for people to understand the critical importance of therapeutic development and how much researchers can accomplish when focused on a singular goal.

Our goal is to cure Parkinson's. And thanks to all of you, we are getting closer. The Michael J. Fox Foundation remains committed to your research and to providing the resources you need to conduct it.

Sincerely,

Todd Sherer

Todd Sherer, PhD CEO, The Michael J. Fox Foundation

About The Michael J. Fox Foundation for Parkinson's Research

As the world's largest nonprofit funder of Parkinson's research, The Michael J. Fox Foundation is dedicated to accelerating a cure for Parkinson's disease and improved therapies for those living with the condition today. The Foundation pursues its goals through an aggressively funded, highly targeted research program coupled with active global engagement of scientists, Parkinson's patients, business leaders, clinical trial participants, donors and volunteers. In addition to funding more than \$900 million in research to date, the Foundation has fundamentally altered the trajectory of progress toward a cure. Operating at the hub of worldwide Parkinson's research, the Foundation forges groundbreaking collaborations with industry leaders, academic scientists and government research funders; increases the flow of participants into Parkinson's disease clinical trials with its online tool, Fox Trial Finder; promotes Parkinson's awareness through high-profile advocacy, events and outreach; and coordinates the grassroots involvement of thousands of Team Fox members around the world.

Stay Connected

We Invite You to Explore Partnering Opportunities and Stay in Touch via Email and Social Media

Receive News and Updates

Sign up to receive e-mail updates on funding opportunities, new resources and relevant news michaeljfox.org

Follow Us



@MichaelJFoxOrg and @TeamFox



linkedin.com/company/michaeljfoxorg



facebook.com/michaeljfoxfoundation

Partner with Us

MJFF is an experienced partner in Parkinson's research and advocacy. The Foundation is open to diverse partnership models including financial, non-financial and in-kind options to collaborate. Whether your company is emerging or established, if you share our goal of helping Parkinson's patients through research and education, we want to hear from you. Learn more about Collaborative Research Opportunities on our website or contact us at researchpartnerships@michaeljfox.org to discuss the full range of partnering options with our staff.



2020 Parkinson's Disease Therapeutics Webinars Series

The Michael J. Fox Foundation (MJFF) sincerely thanks the moderators, panelists, participants, and sponsors who made our inaugural Parkinson's Disease Therapeutics Webinars a success. We designed this free series to foster discussions about new findings and hot topics in Parkinson's research and development. Though the series was initially conceived as a way to bring the research community together during the pandemic, we plan to continue into 2021 after receiving such positive feedback.

An overview of the complete 2020 series follows, along with links to watch each session on demand.

Patient-Focused Drug Development: Novel Instruments to Capture Patient Outcomes in Parkinson's

Tuesday, June 16, 2020

The series kick-off highlighted MJFF-funded research to identify the symptoms and aspects of disease burden that matter to people living with Parkinson's.

WATCH EVENT VIDEO →

Molecular Signatures of Parkinson's Disease

Thursday, July 16, 2020

Kenneth Marek, MD, the principal investigator of MJFF's landmark study, the Parkinson's Progression Markers Initiative (PPMI), moderated this session, which delved into the available data and shared some key insights to date from analysis of the PPMI dataset.

WATCH EVENT VIDEO →

The Many Faces of Parkinson's

Tuesday, September 15, 2020

This virtual "patient day" brought together industry professionals, people with Parkinson's and care partners to share experiences of Parkinson's; its varying onset, progression, and symptoms; and unmet needs.

WATCH EVENT VIDEO →

Where We Are & Where We Are Heading: A Look Ahead at Therapeutic Development in Parkinson's

Thursday, October 8, 2020

This panel covered therapeutic development from identifying novel targets through advancing compounds to the clinic with an emphasis on emerging trends and opportunities to accelerate future development.

WATCH EVENT VIDEO →



Thank You to Our 2020 Sponsors

PARTNER





SUPPORTER











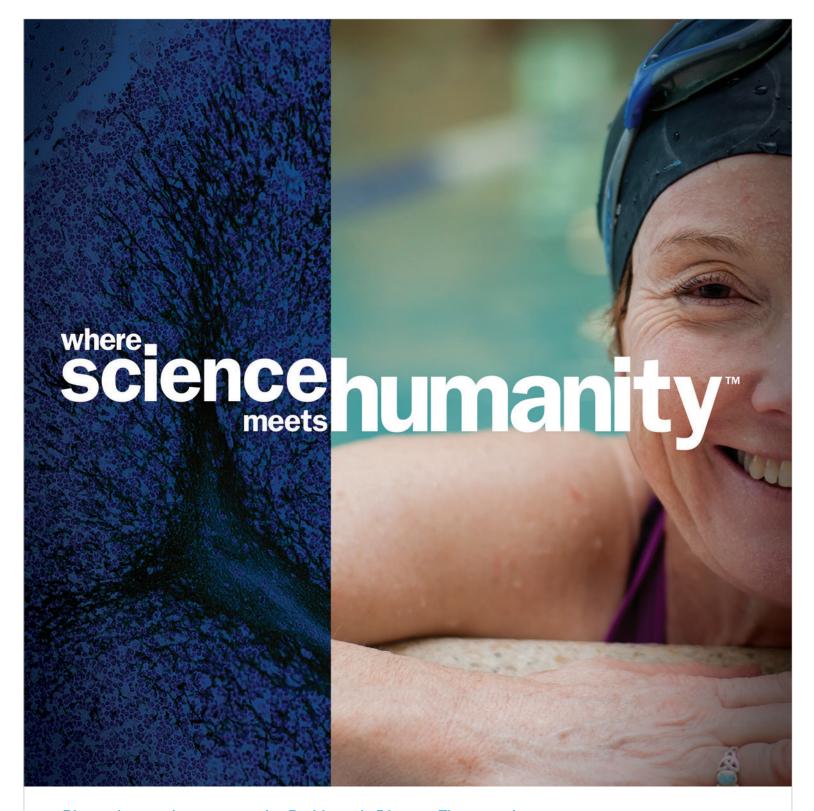


CONTRIBUTOR



If you are interested in 2021 sponsorship opportunities, contact us at researchpartnerships@michaeljfox.org.





Biogen is proud to support the Parkinson's Disease Therapeutics Webinars hosted by The Michael J. Fox Foundation

At Biogen, we are pioneering new science that takes us deep into the body's nervous system, and stretches wide across digital networks and patient communities, to better understand, and preserve, the underlying qualities of our essential human nature.

biogen.com





ENGINÉERING HOPE

Discover how we're using gene therapies to help build a better future for patients with serious diseases.

We are currently developing AAV-GAD, an investigational gene therapy designed to deliver the glutamic acid decarboxylase (GAD) gene directly to a key part of the brain in order to normalize the network that regulates movement. The GAD gene therapy is designed to improve symptoms associated with Parkinson's disease without affecting other areas of the brain and minimizing the likelihood of side effects.





Visit us at www.meiragtx.com



FOCUSED ON DEVELOPING SPECIALTY TREATMENTS

for debilitating diseases that are often difficult to diagnose and treat, providing hope to patients and their families.

Sanofi Genzyme is proud to sponsor The Michael J Fox Foundation for Parkinson's Disease Research 2020 Parkinson's Disease Therapeutics Conference.

www.sanofigenzyme.com

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Sunovion is proud to support The Michael J. Fox Foundation's 2020 Parkinson's Disease Therapeutic Webinar Series

We are a global biopharmaceutical company focused on the innovative application of science and medicine to help people with serious medical conditions, including Parkinson's Disease. Our spirit of innovation is driven by the conviction that scientific excellence paired with meaningful advocacy, research and education can improve lives.



Visit www.sunovion.com and connect with us on:









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Even in the face of a global pandemic, Parkinson's research doesn't stop...

and neither do we.



criver.com/ParkinsonsDisease



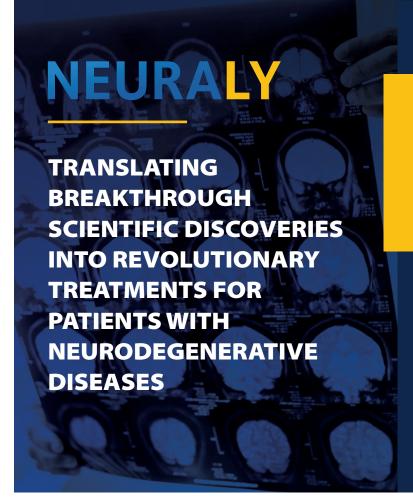
We are UCB.

We are inspired by patients, driven by science.

Building on our heritage in Parkinson's disease and neurology, we are committed to supporting the movement disorder community: today and into the future. Find out more at UCB.com.



www.ucb.com



PRISM EARLY-STAGE PARKINSON'S RESEARCH STUDY

The **PRISM Parkinson's Research Study** is evaluating an investigational drug to determine if it might slow down the progression of Parkinson's disease (PD). Patients who enroll in this study will receive the study drug weekly over a period of 36 weeks.

Study Criteria

To participate in this study, patients must:

- Be 30 to 80 years old
- Have early-stage Parkinson's disease
- Not be on any current treatments for PD
- Not have cognitive impairment

Consider telling your patients about this trial: **888-497-4890** | **PrismPDStudy.com**

The Michael J. Fox Foundation for Parkinson's Research

Founded in 2000, The Michael J. Fox Foundation for Parkinson's Research (MJFF) is the world's largest nonprofit funder of Parkinson's disease research. With no endowment, MJFF immediately deploys non-dilutive capital and intellectual resources with one single-minded goal: to accelerate development of improved therapies and, ultimately, a cure for those living with Parkinson's today.

Our Holistic Approach to Investing

Our internal team of PhDs and MDs and our responsive funding strategy allow us to direct support where it will have the greatest impact on patients' lives. We support the discovery and validation of promising therapeutic targets and invest in translational and clinical projects to actively de-risk the Parkinson's field and attract larger funders. MJFF also provides resources to enable the field to conduct research efficiently and cost-effectively and ensure the best ideas move forward toward regulatory approval and marketability.

"The Michael J. Fox Foundation has managed to become, in its short life, the most credible voice on Parkinson's research in the world."

The New York Times

michaeljfox.org/funding

MJFF Resources



Funding

Apply to open Requests for Applications (RFAs) for research programs

michaeljfox.org/funding



Data and Biospecimens

Access data and biospecimens from several pre-clinical and clinical efforts

michaeljfox.org/data-resources michaeljfox.org/biospecimens



Recruitment Support

Download materials to support recruitment and retention and connect with potential volunteers through Fox Trial Finder

michaeljfox.org/ study-recruitment



Research Tools

Purchase assays, antibodies, models and other research tools from MJFF partners

michaeljfox.org/ toolscatalog



2021 Funding Request for Applications



The Michael J. Fox Foundation is accepting applications for 2021 research support.

Application deadline for all RFAs is

October 22, 2020.

The Foundation will support projects through six funding programs:

Accelerating Publication of Parkinson's Disease Replication Data

Supports publication of replication data through a partnership with the *Journal of Parkinson's Disease*

Analysis of Patient-Reported Outcomes from Fox Insight

Funds analyses of data collected through the Fox Insight study

Development and Validation of Improved Outcome Measures

Funds the development of Parkinson's biomarkers

PIPETTE: 4R Tau and Alpha-Synuclein PET Tracer Development

Funds the development of novel PET tracers for tau and alpha-synuclein

Target Advancement Program

Funds research to validate Parkinson's targets and define pathways

Therapeutic Pipeline Program

Funds therapeutic development in Parkinson's with specific interest in (1) non-motor symptoms and (2) telehealth-based interventions

Visit michaeljfox.org/funding for more on these programs and to download applications. Email grants@michaeljfox.org with questions.





Funding Opportunity for Movement Disorders Fellowship Training

The Edmond J. Safra Fellowship in Movement Disorders offers funding to eight medical centers worldwide to each train a new movement disorder clinician-researcher over a two-year period.

A collaboration between The Michael J. Fox Foundation and the Edmond J. Safra Foundation, this program is designed to grow the global base of movement disorder specialists to care for people with Parkinson's and other movement disorders and lead research into their most pressing needs.



Applications due **December 10, 2020**. Learn more and apply at michaeljfox.org/grant/edmond-j-safra-fellowship-movement-disorders



ASAP Collaborative Research Network Request for Applications

AWARDS: Up to \$9 million for three-year grants

The ASAP Collaborative Research Network is a program of the Aligning Science Across Parkinson's (ASAP) initiative being implemented by The Michael J. Fox Foundation. The program seeks to fund research projects to address key knowledge gaps in the basic disease mechanisms that contribute to Parkinson's development and progression.

Applications must focus on one the following scientific themes:

- + Biology of PD-Associated Genetics
- + Neuro-immune Interactions

The RFA is open to public and private non-profit entities, agencies of the federal government, and for-profit entities. Applications must be submitted by multidisciplinary, multi-institutional research teams consisting of 3 to 5 investigators.

DEADLINE Pre-proposals due:



Portal opens
October 15, 2020

Full proposals will be invited

Apply at www.parkinsonsroadmap.org

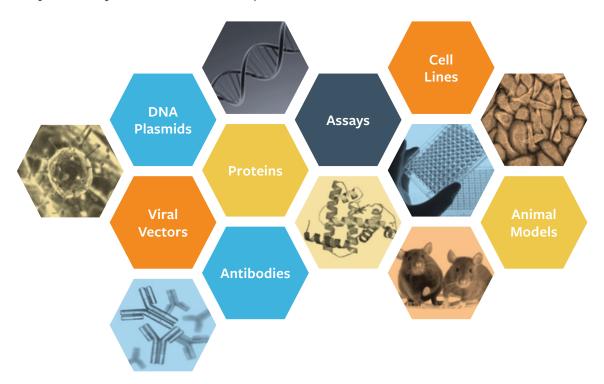
Questions should be sent to grants@parkinsonsroadmap.org

ASAP is leveraging The Michael J. Fox Foundation's grant administration and grantmaking infrastructure to receive applications, administer the review process and make grant awards to projects selected for funding.



Research Tools Catalog

Researchers need well validated, easily accessed tools to generate insights and develop new therapeutics. The Michael J. Fox Foundation is committed to making these available. We collaborate with partners in academia and industry to identify gaps in current tools and then work with appropriate vendors to engineer them at a consistent, high level of quality, and ensure they are easily accessible for widespread use.



Identification

We prioritize the generation of the critical tools that will advance the field based upon current gaps, and suggestions from the research community.

Characterization

We collaborate with relevant partners such as CROs and academic labs to fully examine and validate each tool depending on the end use.

MJFF has 125+ currently available molecular tools and animal models, and 40 more in development.

Generation

We contract with partners including CROs and academic labs to create each research tool.

Distribution

We work with vendors to facilitate the distribution of tools to both non-profit and for profit researchers at low costs with limited restriction.

To access MJFF's research tools, visit michaelifox.org/toolscatalog.





Parkinson's Disease Research Tools Consortium

PARKINSON'S RESEARCHERS RELY ON TOOLS, such as laboratory reagents and pre-clinical models, to understand the causes and mechanisms underlying disease and develop effective therapeutics. The Michael J. Fox Foundation for Parkinson's Research (MJFF) works with contract research organizations to create accessible and well-characterized tools, saving researchers time and money.

The Parkinson's Disease Research Tools Consortium was established in 2013 to leverage the expertise of industry partners to identify and develop the most critical tools to accelerate Parkinson's research. Members of this precompetitive group collaborate with MJFF to prioritize, design, and execute new research tools projects that address unmet research needs. With the support of the Consortium, MJFF is developing over 40 new research tools in 2020. Those already available are listed in our **Research Tools Catalog**.

Industry groups interested in joining the consortium are invited to contact us for more information at researchpartnerships@michaeljfox.org.

Industry Partners











































Fox DEN:



Patient Outcomes at Unprecedented Scale

Data from the Fox Insight study is available to qualified researchers through the Fox Insight Data

Exploration Network (Fox DEN). Fox Insight is an online clinical study with a diverse cohort of people with Parkinson's and age-matched control volunteers. It is now the largest cohort in Parkinson's capturing longitudinal patient-reported outcomes (PROs) from nearly 50,000 volunteers, with plans to recruit more than 100,000. It provides the research community with critical insights into the lived experience, genetics and variability of Parkinson's disease, which can inform decisions on therapeutic development, clinical trial design, health economics and outcomes research, and more.

Included in the Fox Insight schedule of activities are:

Routine Assessments

Survey data collected longitudinally throughout the study

- + Medical history
- + Medications
- + QoL and activities of daily living
- + Motor and non-motor symptoms
- + Physical activity
- + Cognition
- + Environmental exposure

Substudies

Remote, multi-modal data collection

- + **Genetics Powered by 23andMe:** genetic data from up to 17K PD patients in the US
- + More coming soon

Ancillary Studies

Researcher-driven questionnaires

- + **Example:** 1,500 responses from PD volunteers to a novel survey instrument collected in 8 days (over 4,500 responses total)
- + Optional non-routine surveys



Visit **foxden.michaeljfox.org** to learn more. **michaeljfox.org/research**



AMP PD KNOWLEDGE PORTAL



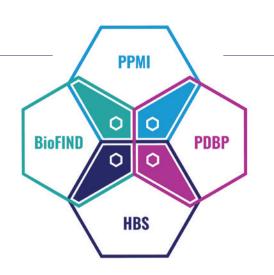
amp-pd.org

Accelerating Medicines Partnership in Parkinson's Disease (AMP PD) leverages well-characterized cohorts with existing biosamples and clinical data collected under comparable protocols and using common data elements. The AMP PD Knowledge Portal enables you to study complex, harmonized data sets and perform genome-wide analyses at a scale previously impossible. By bringing these data together, AMP PD provides a controlled access mechanism for dissection of new targets, disease subtypes, and the identification of predictive markers for disease progression and prognosis.

CURRENT COHORTS

Additional cohorts in development

Parkinson's Progression Markers Initiative (PPMI) Parkinson's Disease Biomarkers Program (PDBP) **Harvard Biomarkers Study (HBS) BioFIND**



AVAILABLE DATA

More data types planned



8,356 **RNA** samples



4,298 **Clinical records** from participants



3,941 **WGS Samples**

AMP PD is a public-private partnership managed through the Foundation of the National Institutes of Health (FNIH)





Bristol Myers Squibb













Longitudinal Data and Biospecimens



The Parkinson's Progression Markers Initiative (PPMI) is a landmark, international clinical study that aims to verify biomarkers of Parkinson's disease progression. At 33 clinical sites in 11 countries, PPMI is collecting imaging, biologic samples and clinical and behavioral assessments from hundreds of subjects for a minimum of five years. The data and biosamples comprise a robust Parkinson's database and specimen bank and are made available to the research community by request at **ppmi-info.org**. PPMI is sponsored by The Michael J. Fox Foundation and funded by a consortium of 32 industry partners.

Included in the PPMI schedule of activities are:

Data

- + Motor Assessments
- + Neuropsychiatric Assessments
- + Neurobehavioral Testing
- + Cognitive Testing
- + Medical and Family History (including Demographics)
- + Environmental Risk Factors
- + Digital Sensor Data

Imaging

- + DaTSCAN
- + Structural MRI
- + Diffusion Tensor Imaging
- + Resting State fMRI
- + VMAT-2 PET
- + Florbetaben PET

Biospecimens

- + Cell lines
- + Cerebral Spinal Fluid
- + DNA
- + Peripheral Mononuclear Blood Cells
- + Plasma
- + RNA
- + Serum
- + Urine
- + Whole Blood
- + Induced Pluripotent Stem Cells
- + Fibroblasts

To achieve its goal of verifying Parkinson's disease progression markers to identify individual measures or a combination of measures that demonstrate interval change, PPMI is studying multiple cohorts of significant interest, including:

Visit PPMI-info.org to access the data and contact researchpartnerships@michaeljfox.org to learn about collaborating on the study.

Study Cohort	Enrollment
De Novo PD	n = 423
Healthy Controls	n = 196
SWEDD	n = 64
Prodromal Focus on Hyposmia & REM Sleep Behavior Disorder	n = 65
Genetics: LRRK2 (Unaffected and PD)	n = 372
Genetics: GBA (Unaffected and PD)	n = 246
Genetics: SNCA (Unaffected and PD)	n = 27



Biospecimens

The Michael J. Fox Foundation makes biospecimens available from select Parkinson's studies for discovery and validation research. Available sample vary by cohort and can include: DNA, RNA, CSF, whole or pellet blood, plasma, serum, urine, saliva, peripheral mononuclear blood cells, fibroblasts, induced pluripotent stem cells, skin tissue, colon tissue, and/or submandibular gland tissue.

You can also view our Biorepository Inventory at **mjffbiobank.org** and our datasets at **michaeljfox.org/data-resources**.

16 Available Cohorts

24-Hour Biofluid Sampling

Cohort Size: ~30

Arizona Parkinson's Disease Consortium

Cohort Size: 450

AVE-8112

Cohort Size: 13

BioFIND

Cohort Size: ~200

CERE-120

Cohort Size: 148

DATATOP

Cohort Size: 800

FS-Zone

Cohort Size: 217

LRRK2 Cohort Consortium

Cohort Size: 3,200

LRRK2 PBMC & Urine Biobank

Cohort Size: ~150

Nilo-PD

Cohort Size: 76

Parkinson's Progression Markers Initiative

Cohort Size: 1,500+

PARS

Cohort Size: 301

S4

Cohort Size: ~150

STEADY PD - 3

Cohort Size: 366

SURE PD - 2

Cohort Size: 75

SURE PD - 3

Cohort Size: 268

The de-identified data included in the specimen catalogs is intended for use in sample selection, ONLY, and may not be shared or used for publication. You will be asked to sign a limited data use agreement prior to being granted access to any specimen catalog. Access to biospecimens and full data sets must be approved by MJFF and/or the NINDS BRAC prior to any sample distribution.

Visit michaelifox.org/biospecimens for more information.



Parkinson's Clinical Trial Companion



A dual-sided educational suite designed to support progress in Parkinson's research by increasing patient participation in clinical studies

Trial Resource Pack for Study Teams

The Trial Resource Pack is geared toward study sponsors, contract research organizations and trial teams, and comprises a manual of best practices and corresponding toolkit of customizable recruitment and retention materials. Using the participant journey as a guiding principle, the Trial Resource Pack equips researchers with tools necessary to optimize clinical trial design and enhance engagement with patient communities.

The Accelerating Clinical Trials Manual includes:

- + **Instructional chapters** to help you develop sustainable recruitment and retention strategies
- + Case studies from past and current Parkinson's studies
- + **Key considerations** for customizing your specific study needs

The Recruitment and Retention Toolkit includes:

- + How-to guides to get you started
- + **Customizable templates** for creating study materials
- + Sample study materials for reference
- + Information sheets to educate participants

Download the full suite at michaeljfox.org/ResourcePack.

Interested in recruitment and retention services? Contact us at trialsupport@michaeljfox.org.

Trial Participant Pack for Study Volunteers

The Trial Participant Pack is a patient-centric suite of materials that illuminate and demystify multiple aspects of clinical research through the use of a 52-page guidebook and complementary videos.

Access the Trial Participant Pack at michaeljfox.org/ParticipantPack.



Sponsorship Opportunities



MJFF is actively planning and executing in the online space, especially during this extraordinary COVID-19 moment, and offering a range of vital opportunities to engage the largest national audience of any organization working in Parkinson's.

Your support will provide best-in-class educational offerings, appealing lifestyle resources and interactive virtual engagements each year that connect your customers, leadership and employees with high-impact programs that are changing lives. Varying programs and levels of corporate sponsorship ensure we can work together to maximize value for your company, whether you're building your visibility within the Parkinson's community or seeking ways to expand brand trust, alignment and preference. These partnerships underpin our shared mission and drive toward improving the lives of those impacted by Parkinson's today.

Multiple high-impact programs allow you to mix and match the value of each programs' goals and opportunities for visibility, with your organizational and corporate culture objectives. In 2020 and into 2021, MJFF will be hosting three flagship programs that cut across two pillars of community engagement:

+ Building Connections within the Parkinson's Community

These programs seek to combat isolation in the face of the COVID-19 pandemic and bring members of the Parkinson's community together.

+ Educating and Empowering the Community to Live Well with Parkinson's

These programs deliver resources and trusted education to the Parkinson's community and empower those affected by Parkinson's to live well.

Further discussion on how one or multiple programs can be tailored to meet your needs is welcomed. If you are interested in learning more about partnering with us, please reach out to researchpartnerships@michaeljfox.org.

