

2021 PARKINSON'S DISEASE THERAPEUTICS WEBINARS

Overview & Resources

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WELCOME

We hope you enjoyed our second Parkinson's Disease Therapeutics Webinar series. The Michael J. Fox Foundation (MJFF) appreciates the value of collaboration and bringing the Parkinson's research and clinician 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 these past two years. We pivoted and met with you virtually and continue to work toward our mission to cure Parkinson's and speed better treatments. 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.

We plan to resume our Parkinson's Disease Therapeutics Conference in person in fall 2022. We look forward to welcoming you back to our annual scientific conference and the only in the world focused exclusively on Parkinson's drug development. We will update you on our plans and we hope to see you there.

Sincerely,

Brian Fiske, PhD, Co-Chief Scientific Officer Mark Frasier, PhD, Co-Chief Scientific Officer

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2021 PARKINSON'S DISEASE THERAPEUTICS **WEBINAR SERIES**

The Michael J. Fox Foundation sincerely thanks the moderators, panelists, participants and sponsors who made the Parkinson's Disease Therapeutics Webinars a success for the second year. An overview of the complete 2021 series follows, along with links to watch each session on demand.

The Promise of Precision Medicine for Parkinson's Disease: Today's Science and **Tomorrow's Trials**

February 25, 2021

In our first event of the year, panelists discuss clinical heterogeneity in Parkinson's disease; using genetic, biologic and pathologic signatures for designing clinical trials; and precision medicine approaches for Parkinson's.

Watch Event Video (>)



Using Patient Voices to Transform Your Parkinson's Research

June 29, 2021

Panelists discuss why it's critical to bring patients and care partners into the research & development process early and often as well as how to think about and design clinical trials with a patient-centered lens.

Watch Event Video (>)



Centering Equity and Inclusion to Advance PD Therapeutic Development

September 14, 2021

Panelists highlight evolving initiatives — including those compiling genetic insights from diverse global cohorts — that can provide a more complete understanding of Parkinson's and fuel novel avenues for therapeutic development.

Watch Event Video (>)



Diverse Approaches Accelerating PD Target and Drug Screening

November 9, 2021

Leaders from academia and industry highlight technologies that are fueling advances in Parkinson's drug discovery.

Watch Event Video (>)



Progress in the PD Pipeline: A Snapshot of MJFF's Funded Programs

November 10, 2021

Panelists showcase novel translational and clinical research from MJFF's research portfolio including new and unpublished results.

Watch Event Video (>)







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

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We are currently developing AAV-GAD, an investigational gene therapy designed to deliver the glutamic acid decarboxylase (GAD) gene to the brain in order to normalize motor circuits and improve symptoms associated with Parkinson's disease without affecting other areas of the brain.



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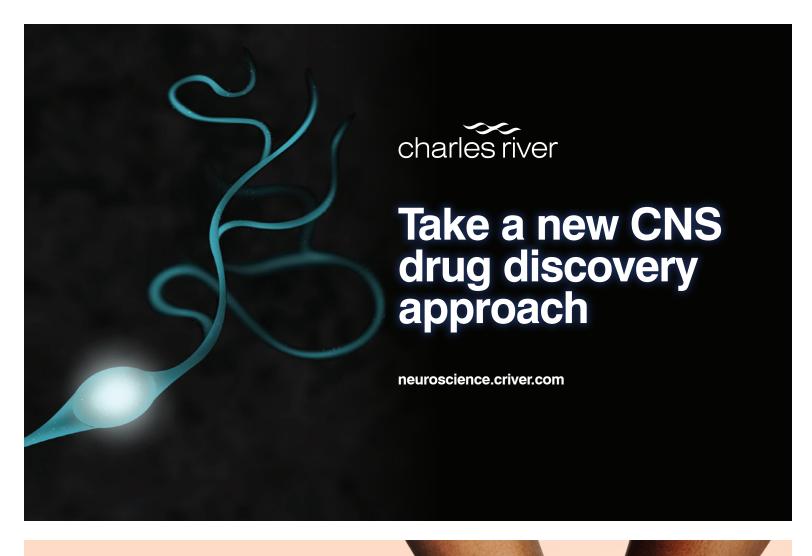


The **PRISM Parkinson's Research Study** is evaluating an investigational drug to determine if it might slow down the progression of Parkinson's disease (PD). Patients who enroll in this study will receive the study drug weekly over a period of 36 weeks.

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



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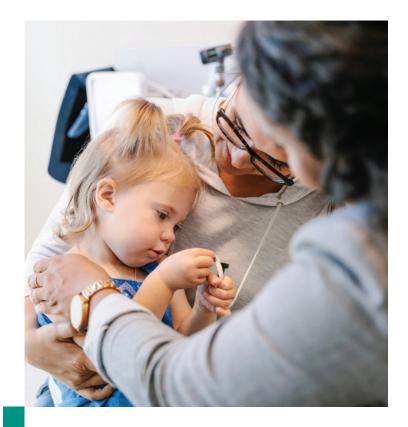
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BBK Worldwide is proud to support The Michael J. Fox Foundation Parkinson's Disease Therapeutics Webinars Series.

Passionate in the effort to advance Parkinson's disease research, we create meaningful engagement at key touchpoints in the clinical trial patient journey.



From patient outcomes To research breakthroughs And new treatments that matter



We believe that the patient should always come first. That's why we do things differently at IQVIA – by bringing the science of healthcare together with patient experience, clinical outcomes-based strategies and expert scientific knowledge. It's how we look beyond what's expected to see what's possible.



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The Michael J. Fox Foundation 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, Foundation 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

Funding programs will open in early 2022.

+ The 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

Applications due December 9, 2021.

View our open funding programs and application deadlines at michaeljfox.org/funding.

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



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 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 \$1.5 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

"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.

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 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 booklet 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. There are over 250 advisors that help 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 National Institutes of Health. 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.





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.

Clinical Data 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 and biosamples from well-characterized clinical 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.

MJFF and Digital Health

MJFF is committed to supporting advances in medical devices that have the potential to help the Parkinson's community. If you are a device developer interested in learning more about opportunities to collaborate with MJFF, please reach out to researchpartnerships@michaeljfox.org.

Research Tools Catalog

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.

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**.

Parkinson's Disease Landscape Reports

Over two decades working in Parkinson's alongside leading experts has positioned MJFF to refine and share its perspective on the research landscape. MJFF actively tracks key developments in the Parkinson's landscape, from advancements in basic science research to drugs entering late-stage clinical trials. We summarize key trends, identify gaps, and highlight areas of focus moving forward — not just for the Foundation itself, but to share out with the broader research community.

- + **Clinical Pipeline Report** provides a snapshot of drugs in clinical development, along with high-level perspectives on priority targets and modalities; as well as being an excellent primer for those newer to working in Parkinson's, this resource is a useful tool for anyone seeking a synthesis of Parkinson's clinical development
- + **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 Health Economics and Outcomes Research teams to assess the potential economic benefit of novel Parkinson's therapies

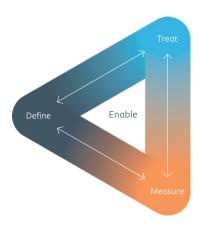
For more information: Download the **2019 Economic Burden of Parkinson's Report**. To request access to the Clinical Pipeline Report, please email **researchpartnerships@michaeljfox.org**.

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
- + **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 four of dozens of projects that have received follow-on funding after MJFF de-risking grants:

Biotech 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.]

AFFIRIS and AC Immune announced a partnership that will advance a vaccine to lower levels of Parkinson's protein alpha-synuclein. (MJFF granted AFFIRIS nearly \$3.5 million for the Phase I trial and follow-up trials of boost vaccines. MJFF also granted AC Immune more than \$5 million since 2015 to develop an alpha-synuclein tracer.)



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 will grow to 50+ sites and follow more than 4,000 participants including 2,000 with Parkinson's risk factors. In 2021, PPMI launched an online platform open to anyone over 18 living in the United States. Refer your patients, share with your network and sign up yourself. Learn more.

PPMI is sponsored by MJFF with support from nearly 40 industry, nonprofit 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

1,400+

study participants enrolled

4,000+

participant recruitment goal in clinic

100,000+

participant recruitment goal online

8.5 million+

downloads of PPMI data by researchers

35+

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.

Parkinson's Research Tools Consortium

MJFF and industry collaborators work to identify, develop, characterize and validate new preclinical 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: Members recommend and provide strategic direction for the development of new research tools while saving time and money by engaging in regular discussions with MJFF and other experts.

Commitment: Financial commitment required.

Learn more about the Parkinson's Research Tools Consortium.

Knowledge-sharing Consortia

Join interactive, dynamic discussions with fellow experts working in similar areas of Parkinson's research to regularly discuss methods and findings, identify challenges to progress, and propose resources and initiatives to overcome those hurdles. Active knowledge-sharing consortia focus on the following priority therapeutic targets or pathways: Alphasynuclein, GBA, Inflammation, LRRK2, Parkin/PINK1.

Commitment: Complimentary membership for MJFF grantees and other researchers willing to share prepublication data in a confidential setting.

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 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 is required for the conference. Other meetings and workshops are typically free to attend for invited guests.

Webinars for Researchers

MJFF offers free webinars on topics of interest to researchers, clinicians and industry professionals.

Previous webinars — all available anytime on-demand — have covered topics such as our funding strategy and programs, molecular signatures of disease and novel strategies to capture patient outcomes.

Commitment: Thanks to the support of our sponsors, webinar attendance is available at no cost.

Learn more about webinars.





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.

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 hand-in-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: Please email **researchpartnerships@michaeljfox.org** to learn 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



Partnerships are critical to our mission. We work with biotechs, pharma and health tech companies to advance our shared goals through funding, in-kind contributions, strategic input and more. Below we highlight partners that support Foundation programs and initiatives.

Funding Partners in Premier Consortia



Parkinson's Progression Markers Initiative

4D Pharma AbbVie Inc.

AcureX Therapeutics

Allergan

Amathus Therapeutics

Aligning Science Across Parkinson's (ASAP)

Avid Radiopharmaceuticals

Bial Biotech Biogen BioLegend

Bristol Myers Squibb Calico Life Sciences LLC Celgene Corporation DaCapo Brainscience Denali Therapeutics

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Sanofi Genzyme

Servier

Takeda Pharmaceutical Company

Teva Neuroscience, Inc.

UCB

Vanqua Bio

Verily Life Sciences

Voyager Therapeutics, Inc. Yumanity Therapeutics



Parkinson's Disease Education Consortium

AbbVie Inc.

Amgen

Amicus Therapeutics

ApoPharma

AstraZeneca Pharmaceuticals

Biogen

Bristol Myers Squibb

Eli Lilly and Company

Lundbeck

Merck & Co., Inc.

Mission Therapeutics

Pfizer

Orion Pharma

Orphazyme

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Abbott

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Acorda Therapeutics

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

The New York Times

 $\$1.5\,BILLION+$

"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

Research Partnerships Team at MJFF | researchpartnerships@michaeljfox.org

