

The need for
new Parkinson's
disease treatments
**has never been
more urgent.**

THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH



NEARLY FIVE MILLION PEOPLE WORLDWIDE ARE LIVING WITH PARKINSON'S

disease, making it second only to Alzheimer's disease in prevalence. According to the Centers for Disease Control, Parkinson's disease is the 14th leading cause of death in the United States. There is no known cure. And 60,000 new U.S. cases will be diagnosed this year alone (a figure that will continue to climb as more baby boomers reach retirement age).

The Michael J. Fox Foundation (MJFF) exists for one reason: to accelerate the next generation of Parkinson's disease treatments. In practice, that means identifying and funding projects most vital to patient impact; spearheading problem-solving around seemingly intractable fieldwide challenges; coordinating and streamlining the efforts of multiple, often disparate, teams; and generally doing whatever it takes to drive faster knowledge turns for the benefit of every life touched by Parkinson's disease (PD).

FUNDING BASIC SCIENCE ALONE IS NOT ENOUGH

Public and private investments worldwide continually grow the base of human understanding of biology; newspapers report on intriguing research discoveries every week. But the process of translating these findings into medical treatments is slow and inefficient. The primary drug therapy for Parkinson's, levodopa, was a breakthrough when it was discovered in the 1960s. Unfortunately, this drug — which does nothing to slow or stop the progression of the disease, treats only some of the symptoms patients experience, becomes less effective over time, and causes disabling side effects — remains the "gold standard" Parkinson's treatment in 2017.

Since its founding in 2000, The Michael J. Fox Foundation has worked to jolt Parkinson's drug development out of this standstill. Our activities go far beyond funding basic research discoveries. We identify and fund truly innovative ideas in PD discovery (vetting over 600 Parkinson's drug targets to date), including high-risk, high-reward opportunities that other funders can't or won't take on. Sadly, the reality of science is that most of these ideas will fail. That's why we laser-focus on the relatively few targets that show real potential to help people, and proactively

chaperone them along the pipeline toward clinical trials. At the same time, we put building blocks in place to increase the odds of clear, actionable results from those trials. Positioned at the global hub of Parkinson's research, we strive to set the standard for impact and accountability.

PROBLEM-SOLVERS IN PURSUIT OF A CURE

Drug development for brain disease is a complex ecosystem. Identifying the most promising targets and the most critical problems is hard work in itself. MJFF's on-staff research team, extensively trained in neuroscience and project management, prioritizes the most important projects, articulates their importance, gains stakeholder buy-in, and provides funding and technical assistance to push them forward. Our expertise in science and the business of science helps us solve systemic challenges standing in the way of progress. For example:

- » Few incentives or natural "handoffs" are built into the scientific process to allow academic and industry researchers to communicate and work together to advance treatments.
- » With no Parkinson's biomarker, clinical studies lack objective endpoints, leading to drawn-out timetables, high costs and

20



MJFF "de-risking" successes that have received significant follow-on funding

100



Clinical trials supported by MJFF to date

600



Drug targets vetted and prioritized through MJFF funding

inconclusive results — plus a nearly insurmountable hurdle to regulatory approval, especially for disease-modifying drugs (patients' greatest unmet medical need).

- » Industry funding for diseases of the brain, critical to the clinical trial economy and the regulatory approval process, is growing increasingly risk-averse as costs climb.
- » Clinical trials are hampered by a chronic shortage of volunteers. Estimates hold that 85 percent of trials are delayed by recruitment challenges; up to 30 percent fail to enroll a single participant.
- » Treatment outcome measures are too often developed by researchers with little or no input from patients to ensure that drug development is informed by individuals' actual experience of disease.
- » While "patient engagement" is a buzzword, Parkinson's patients are often isolated, with few sources of information on how to engage. Many never even make it to see a movement disorder specialist (a neurologist with specialized training in Parkinson's disease).

SETTING THE AGENDA

Our various initiatives to make inroads against these challenges have positioned our Foundation as a trusted strategic leader and partner to researchers, patients, thought leaders and

philanthropists. With an unbiased expert perspective and a global network of contacts, our wide-ranging view of the field lets us target resources where they are needed most — to pay off for patients faster. We:

- » Review close to 1,000 grant proposals each year and fund millions in grants worldwide.
- » Maintain a formal and informal dialogue with a global who's-who of PD experts, regularly convening conferences, meetings and workshops to push through setbacks, build field consensus around the most important projects and drive next steps.
- » Sponsor landmark clinical studies — an unusual role for a group like ours, more commonly played by government institutions or pharmaceutical firms.
- » Mobilize the patient community to get involved in research, and develop technology-enabled platforms that provide appealing entry points for patient engagement.
- » Bring thousands of leading academic and industry Parkinson's researchers from around the globe to participate in our consortia, assess and review grants, and make use of our open-access data and research tools.
- » Gather specialized input from both researchers and patients to inform our program strategy.

A CURE IN OUR LIFETIME

The New York Times has called The Michael J. Fox Foundation "the most credible voice on Parkinson's research in the world." We operate according to best practices and remain accountable for ensuring the highest possible degree of effectiveness. With leadership recruited from the world of finance, management and neuroscience, we bring strategy, scientific and operational expertise, and an investment professional's sensibility to the management of the Foundation. The team pursues an entrepreneurial model focused on performance and outcomes, which includes a non-traditional view of return on financial capital. Our deliberate lack of endowment or excessive reserves is emblematic of our goal to "go out of business." And we constantly monitor costs to maximize the value of donations. As a result, since our founding, 89 cents of every dollar we have spent goes straight to our research programs.

TO LEARN MORE, PLEASE VISIT WWW.MICHAELJFOX.ORG.

14TH



Parkinson's disease is the 14th leading cause of death in the U.S.

\$19.8 – \$26.4B



Estimated annual economic burden of Parkinson's disease

89



Cents per dollar spent at MJFF directed toward research programming





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Grand Central Station, P.O. Box 4777
New York, NY 10163-4777

1.800.708.7644 | michaeljfox.org