



THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

ACCELERATING THE CURE

The newsletter for friends and supporters of
The Michael J. Fox Foundation for Parkinson's Research

WINTER 2012

MARK SELIGER



Getting Drug Candidates into the Clinic

By Seizing the Risk, MJFF Helps Awardees Secure Funds Needed to Develop Novel Parkinson's Drugs

The path to new drugs often begins with a Eureka moment — when an academic working in a lab discovers a specific molecular process in our cells that seems to be causing a disease or its symptoms. It's a key first step toward developing drugs that work by targeting such processes.

For Jeff Conn, PhD, of Vanderbilt University, that stroke of brilliance came in 1998, when his team identified a glutamate receptor called mGluR4 that, when activated, seemed to reduce the activity of a particular part of the brain that becomes overactive in people with Parkinson's disease (PD).

The "aha" moment for Jim Surmeier, PhD, of Northwestern University, came in 2006. His research led him to theorize that calcium channel blockers (such as certain high blood pressure medications, or new drugs like them) might slow or even stop the progression of PD.

Hurdles and Potholes

Unfortunately, while discoveries like those made by Conn and Surmeier provide the genesis for most new therapies, they're merely the first step along a drug development road lined with myriad hurdles and potholes. On average, the time it takes to bring one new drug to market is 15 to 30 years, at a cost of \$1 billion. And this is for those few compounds that actually make it to pharmacy shelves. Most potential treatments die early, in what is known as the "Valley of Death," that period of research when an idea born in a university setting loses steam, and is unable to secure the necessary support from the pharmaceutical industry to move research from the laboratory and into the clinic.

Conn and Surmeier, like most academic researchers in their shoes, knew that without a push from the right source, their ideas risked falling prey to this challenging system. They knew that their novel drug targets might die before they ever exited the Petri dish.

So both scientists, searching for the right engine to drive their research forward, came to MJFF.

And the Foundation was willing to take a risk on both, stepping in where other funders wouldn't.

Great Potential and Calculated Risk

"When we came to MJFF, we had a compelling idea for a Parkinson's drug based on basic science," says Conn. "But it was way too early on for any company to invest in our approach. We needed another source to help us test our hypothesis, to make it more attractive to industry investment. MJFF was that source."

In reviewing Conn's science, MJFF saw great potential, and was willing to take a calculated risk to fund studies that could drive his approach

FDA-approved, it was quickly readied for clinical testing), ultimately, improved compounds that target PD specifically, such as Surmeier's, may be needed.

"Without The Michael J. Fox Foundation, we never would have been able to move forward with the drug discovery effort," says Surmeier.

New Opportunities and Next Steps

In 2012 the payoff of MJFF investment has been huge, for both scientists and their teams. In September, Vanderbilt announced a next step collaboration with Bristol-Myers Squibb for the ongoing development of mGluR4-targeted drugs.

"Establishing this partnership is proof that MJFF can have a huge impact. We are very hopeful that these drug candidates will go the distance." — Jeff Conn, PhD

closer to testing in humans. Since 2005, MJFF has invested nearly \$5 million in Conn's pursuit of a potential new class of PD drugs targeting mGluR4.

Like Conn, Surmeier came to the Foundation for support of pre-clinical studies based on the premise that calcium channel blockers might slow the progression of PD. The data was promising. Surmeier has now identified a new drug class that blocks the calcium channels he believes are responsible for stressing dopamine neurons.

A Phase II clinical trial, in which MJFF invested \$2.1 million for an existing calcium channel blocker called isradipine, recently returned positive safety results. This study team, led by Tanya Simuni, PhD, also of Northwestern University, is hoping to begin a Phase III efficacy study of isradipine in 2013.

Surmeier is optimistic about both isradipine and his new approach, which could provide an additional option for people with PD. While isradipine provided researchers with a fast way to test his theory (since the drug is already

While it is impossible to predict how quickly the program will advance from this point, Conn is hopeful that an experimental drug could enter clinical testing as soon as 2013.

Surmeier has received significant government funding to move his new compounds closer to the clinic. In September, he was awarded one of only three multi-million dollar Blueprint for Neuroscience Research grants from the National Institutes of Health (NIH).

While there's a long road ahead, Surmeier is hopeful that the NIH monies will allow him to move his drug through the Valley of Death toward a Phase I clinical trial in PD patients.

"If we can do this," he says, "I'm sure that the necessary investment of industry will follow."

"My hat is off to the Foundation," says Conn. "Establishing this partnership is proof that MJFF can have a huge impact. We are very hopeful that these drug candidates will go the distance. Without the Foundation, they never would have even gotten off the ground."

THE SHERER REPORT



In 2012 we saw significant progress and change in Parkinson's disease research, despite a climate of insufficient funding. Through the generosity and participation of so many in the Brin Wojcicki Challenge, we are gaining momentum.

Todd Sherer

Todd Sherer, PhD, CEO

Read previous issues at michaeljfox.org/TheShererReport.

Systemic challenges such as governmental budgetary constraints, low risk tolerance from private investors, high clinical trial costs, and non-standardized trial methodology continue to hang over the field. But thanks to patients and supporters like you, our Foundation is spearheading innovative, proactive solutions to the hurdles that stand in the way of bringing new drugs to market.

In September, I was honored to be appointed to the advisory council for the National Center for Advancing Translational Sciences (NCATS), a nascent National Institutes of Health (NIH) initiative. Importantly, it's the first U.S. agency exclusively dedicated to fostering translational research. Last spring, I testified before Congress on the need for NCATS funding and lessons learned from our patient-centric model to speed translation. Also in September, I was invited by the Presidential Council of Advisors on Science and Technology to be part of the release of their recommendations to the President on cooperative efforts around drug development.

The field has uniformly admitted that we have a drug development crisis on our hands — we must do things faster, smarter and more collaboratively. And we must be more cost-effective in our strategies. As we have always believed at MJFF, patients should not have to wait years for relief.

I'm cautiously optimistic that federal efforts are finally under way to improve government capital allocation and research efficiency,

and that such improvements could accelerate the development of new treatments across all diseases in the long run.

But even as we herald these government shifts, MJFF cannot — and will not — slow our pace. We continue our aggressive work to accelerate the development of PD treatments. In 2012, we witnessed key successes from our “doing whatever it takes” strategy, which will continue to drive forward critical projects in 2013:

- As you read in the cover story, Jeff Conn, PhD, and colleagues at Vanderbilt University announced a substantial partnership with Bristol-Myers Squibb (BMS) to move a new symptomatic treatment for PD closer to patients. MJFF has supported Conn's work targeting the glutamate system since 2005, and our sustained high-risk investment garnered the relevant data to entice BMS to bring the program through clinical development. The partnership marks an additional major industry player that has entered the PD drug development arena. Conn is hopeful his drug candidate could enter the clinic as soon as this coming year.

- The MJFF-led Parkinson's Progression Markers Initiative — launched in 2010 — continues to produce results, showing that productive collaboration is possible if someone is willing to take the lead. Working with 12 partners from the pharmaceutical industry to find biomarkers for PD, significant investments to improve participation are bearing fruit, as enrollment is nearly complete. Data made available to researchers worldwide has been downloaded over 30,000 times. In the year to come, we plan to

expand upon findings from PPMI into new efforts studying at-risk populations for PD. (Read more on p. 3.)

- The MJFF initiative to put laboratory tools into the hands of as many researchers as *quickly* and *inexpensively* as possible is also showing impact. Today, seven MJFF-owned pre-clinical models are available at low or no cost to researchers who need them for promising PD drug development studies — and this number will more than double by May 2013. To date, these models have been distributed more than 2,750 times.

- Fox Trial Finder, our Web-based portal unveiled in 2011 to address the challenge of participant recruitment for clinical studies, surpassed our launch goal of 10,000 registrants. This is already translating into results, as clinical trial coordinators have reported a critical boost in recruitment activity. Our new goal is to reach 15,000 volunteers by year-end and 25,000 in 2013. Become one of these volunteers to get new therapies into patients' hands — complete your profile today at foxtrialfinder.org.

These are just a few examples of the important impact that all of us, working together, can make in accelerating PD therapies. But we are not done. We have ambitious plans for 2013 — to continue to translate a growing knowledge of PD genetics into treatments, to develop ways to identify people before they get PD, to engage with industry players to keep them focused on PD, and to launch an ambitious initiative to expand the role patients can play in providing answers to critical research questions. Next year alone, we are striving to fund \$60 million in research.

As always, I am grateful for your dedication to our mission. We are seeing clear progress, but this is not the time to take our foot off the pedal. I'm excited by the distance we have come, and energized by where we will be — thanks to your continued commitment to be part of the answer.

The \$50-million Brin Wojcicki Challenge ends this month, and we need your help to raise the final few million. Your willingness to step up today has never been more important — to the Foundation and Parkinson's patients everywhere.

I hope we can count on you to help us leverage these gains in 2012 and make groundbreaking opportunities in PD research a reality.

GENETICS UPDATE

Nearly 10,000 Strong, 23andMe PD Research Community Drives Genetic Research toward Potential New Drugs



In spring 2009, personal genetics company 23andMe, a Michael J. Fox Foundation research partner, announced an effort to recruit 10,000 people with Parkinson's for genetic research in PD. Today the 23andMe PD Research Community has become the largest single Parkinson's community for genetic research worldwide, comprising more than 9,300 people with Parkinson's across 49 U.S. states and 26 countries.

23andMe allows patients to participate in research from their home computers through a free lifetime membership. Members send in a saliva sample for DNA testing, then fill out surveys online.

There are many reasons to push forward genetic studies of PD. One is to develop ways to predict an individual's risk for developing the disease. Another is to better understand molecular processes underlying PD, in both genetic and non-genetic cases of the disease. This could lead to improved drugs for all patients, not just those who carry genetic mutations.

New research directions

"In the search for causes of PD, it's increasingly clear that many roads lead to Rome," says Emily Drabant, PhD, research development manager at 23andMe. "If we understand what these roads look like, we may be able to develop treatments to stop the disease."

Within its first two years, 23andMe not only confirmed 20 genetic mutations implicated in PD, but also discovered new genetic mutations linked to PD. By combining genetic testing with online reporting, 23andMe arrived at similar conclusions as researchers from a traditional clinical setting. It was a critical proof point validating their "direct-to-patient" approach early on.

23andMe is now moving to tackle increasingly complex questions about PD, including the role of genetics in disease onset.

A personal decision

Participants in the Parkinson's community are screened for 10 validated genetic variants linked to Parkinson's disease risk. However, Drabant notes that it is entirely up to individual volunteers to choose how much to learn about their own Parkinson's genetic status. While all community members receive detailed, personalized genetic reports, individuals can opt not to view these reports. Regardless of this decision, all volunteers can fill out online surveys and fully participate in the scientific progress the community enables.

Clearly, the decision to have one's genome tested is a nontrivial and personal one that can affect the entire family. Given our current understanding of PD, knowing genetic risk won't inform an individual's treatment regimen, and there is no current action plan for preventing PD or treating it differently based on the DNA card you've been dealt.

Still, taking part in genetic research, especially for families with a history of Parkinson's, can be critical to accelerating new and better treatments for the disease. Whether it's through a modern service like 23andMe, or signing up for genetic studies in a traditional research setting, your participation makes a difference.

Gary Schmitz of Grapevine, Texas, didn't think twice about participating. "For me, the question was, why wouldn't you want to contribute?" he says. An early member of the 23andMe PD Research Community, he values the information he has gained. "If you know you're at risk for something, you can do something about it."

To join the 23andMe PD Research community, visit 23andme.com/PD. To learn more about how to get involved in other genetic studies, create a profile at Fox Trial Finder (foxtrialfinder.org), the Foundation's clinical trial matching tool. (If you know your genetic status, be sure to answer the question about genetic testing.)

BIOMARKER UPDATE

New Biomarker Study Seeks People Who Have Had PD for at Least Five Years

To keep the biomarker search moving forward, and to provide science with the most shots on goal as possible, it is critical to continue to identify possible markers. The outcome is a first-of-its-kind collaboration called BioFIND, a multi-site study exclusively focused on identifying entirely new PD biomarkers, driven by MJFF and the NIH National Institute of Neurological Disorders and Stroke (NINDS). Once discovered, these leads will be seamlessly integrated into the Parkinson's Progression Markers Initiative (PPMI) to be validated for PD. MJFF will invest about \$1 million and play an active role in the day-to-day management of the study, and NIH will provide the biorepository.

While both PPMI and BioFIND seek controls to participate, there's an important difference in terms of those PD patients who can participate in this study: PPMI engages those who have been newly diagnosed with PD; BioFIND, however, is searching for people who have had PD for at least five years, and for no more than 15 years. (They should also be between the ages of 55 and 85.)

Since its inception, MJFF has invested \$65 million in the pursuit of a Parkinson's biomarker — a substance, process or characteristic associated with the risk or presence of the disease. Finding consistent Parkinson's biomarkers would allow scientists to predict, diagnose and monitor the disease, and determine which medications might work and which won't. A biomarker would be an invaluable tool to the development of new treatments for patients.

In 2010, MJFF launched PPMI. With enrollment of patients and controls expected to be complete in early 2013, more than 30,000 data downloads have been made for studies across the globe. Today, many of these associated studies are aimed at validating potential biomarkers that researchers have identified in the lab. PPMI has provided the infrastructure for the first multi-site corroborations of observations culled from previous studies conducted at single sites.

PPMI: Delving Deeper into the Pre-motor Symptoms of PD

It's been a big year for PPMI. In the ongoing search to better understand what's happening in the body prior to the motor symptoms of PD, the study is launching a new arm that will include adding a pre-motor cohort.

Back in July at the Movement Disorders Society annual meeting, scientists debated, 'Is PD even rightly defined as a movement disorder anymore?' Here's why: By the time a person is diagnosed with Parkinson's, the disease is already well developed — typically an individual has already lost a significant amount of their dopamine neurons. More and more, research is pointing to the fact that Parkinson's may involve other systems in the body, beyond the central nervous system.

"I'm amazed to think how our vision of PD continues to evolve," says Maurizio Facheris, MD, MSc, associate director of research programs at MJFF. "It has now moved way beyond the substantia nigra [the region of the brain where the dopamine deficit associated with the motor symptoms of PD occurs], even beyond the entire brain itself to include the colon and other systems. We need to expand our thinking in developing treatments that could possibly target systems in the body that we never expected to target."

This new arm of PPMI focuses on three particular study groups that could lead science to the kind of expanded vision that Dr. Facheris is talking about, and, in turn, to treatments that more holistically address the disease. The study will evaluate and follow people who don't have Parkinson's but may be at risk to develop PD over time, including: people who suffer from a reduced sense of smell; those with rapid eye movement sleep behavior disorder (RBD); and those with a mutation in the LRRK2 gene (the single greatest genetic contributor to PD known to date).

If scientists can learn more about the biological processes taking place in people with any of these three conditions, they may be able to define biomarkers at an even earlier stage. This is vital for Parkinson's drug development. Such knowledge could also lead to new targets for drugs to attack the disease before it ever starts.

TO LEARN MORE ABOUT BIOFIND, VISIT FOXTRIALFINDER.ORG/BIOFIND. MORE INFORMATION ABOUT PPMI CAN BE FOUND AT MICHAELJFOX.ORG/PPMI.

GET INVOLVED

...IN THE CHALLENGE

“Sticking My Neck Out for the Right Reasons”: Celebrity Chef Chris Cosentino Wins “Top Chef Masters” — for MJFF

On July 25, alongside nine other award-winning chefs, Chris Cosentino, executive chef at San Francisco's celebrated *Incanto* restaurant, debuted on Season 4 of Bravo's "Top Chef Masters." In addition to the chance to prove his mettle in one of the world's most-watched cooking competitions, his participation on the show meant the opportunity to win \$100,000 or more for the charity of his choice. Chris chose to compete on behalf of The Michael J. Fox Foundation for Parkinson's Research and use his passion for food to help speed a cure for Parkinson's disease.

In the September 26 finale, Chris was crowned 'Top Chef Master.' His winnings for the Foundation topped \$141,000 — which will be doubled to an astonishing \$282,000 by the Brin Wojcicki Challenge!

The Foundation spoke to Chris about his connection to Parkinson's disease, what went into the decision to support MJFF, and the chance TV night with his son that played a role in his choice.



Chris Cosentino

MJFF: What is your connection to Parkinson's disease? How has it affected your life?

Chris: My uncle, John Cosentino, had Parkinson's disease, and it was really eye-opening for me to see how it affected him over the course of 30 years. He was such a gregarious person. My parents divorced when I was young, and growing up we spent a lot of family holidays at his home. He even taught me how to play pool in the basement.

While he wasn't affected mentally by the disease, which was a blessing, his physical symptoms became increasingly debilitating. It limited his ability to practice law, which was his passion. It made me think about what it would be like if I could no longer cook, how devastating that would be. He also was always thinking of what he could do for others. He participated in clinical research during his life because he believed strongly that it was one of the best ways he could help others with this disease.

He passed away two years ago from disease-related complications. Even after his death, he helped others by donating his brain to science. I wanted to follow through with that legacy of selflessness. For me, appearing on Top Chef was

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An Insider's View on the Challenge

An update on the Brin Wojcicki Challenge from Sheila Kelly, MJFF's vice president of development, on what it's making possible, its progress, and how you can help.

How did the Challenge come about?

Sergey Brin, co-founder of Google, and his wife, Anne Wojcicki, co-founder of personal genetics company 23andMe, are longtime Foundation friends and our largest donors for six years running. They believe in our high-risk, high-reward approach to identify the most promising therapies and chaperone them along the pipeline toward clinical testing and patients. To get more people involved to help speed a cure for PD, they challenged us to raise \$50 million from donors like you by December 31, 2012, which they will match dollar for dollar.

What is the Challenge enabling the Foundation to do?

We're using Challenge dollars to move several of the most promising treatments forward at the same time, and to fund research that would otherwise go unfunded. These capacity-building funds also enable us to tackle the significant clinical testing roadblocks therapies face that can slow their progress in getting to patients.

What have you found most inspiring about the Challenge?

I have been amazed by the sheer number of people who have stepped up for the Challenge, and the creative ways in which they're helping us to raise these funds. We've seen a big surge in new donors making an impact through gifts of all sizes. Also, some of our longstanding donors have really dug deep to increase their support and take advantage of the match.

Do smaller gifts count toward the Challenge?

A gift of any amount can count toward the Challenge! And these contributions really add up. In 2011 alone, 17,232 new donors gave \$100 or less to the Foundation. Combined, these gifts totaled over \$1 million — which was doubled to more than \$2 million. Every dollar gets us that much closer to a cure for Parkinson's.

How close are you to meeting the \$50-million goal?

Thanks to the generosity of our friends and supporters around the globe, we've seen a remarkable response to the Challenge. But we still need your help to earn the final few million.



Sheila Kelly

Why does the Challenge come at such an important time?

Today we see before us the most robust therapeutic pipeline for Parkinson's disease in decades, if not ever. No fewer than a dozen recently discovered drug targets are now in human clinical testing for Parkinson's. They represent possible breakthroughs in our ability to address previously untreated symptoms, such as mood disorders and cognitive dysfunction, as well as drug side effects such as dyskinesia (uncontrollable movements). Even more importantly, some of these targets hold potential to meet patients' most critical need: a treatment that could slow or stop disease progression. At a moment like this, we simply can't afford to leave any Challenge dollars on the table.

It's not too late to join the Challenge!
Learn more and make your gift today at michaeljfox.org/challenge.

Creating Community: One Family Rises to the Challenge

a great way to honor his memory and keep him close. I felt like he was there with me during the competition.

In addition to my uncle's experience, a close mentor of mine also has Parkinson's, and it so happened that a number of friends had parents with the disease. When I asked myself, 'What am I willing to stick my neck out for?' I knew that if I could support the Fox Foundation's efforts to speed a cure for this thing, I was sticking it out there for the right reasons.

MJFF: Are you a fan of Michael J. Fox?

Chris: Of course! But it's funny, Michael influenced my decision to compete for the Foundation in a really chance way. I was watching TV with my son, who's seven. *Back to the Future* was on, and right after, he flipped to another channel and there was an episode of "Scrubs" featuring Michael [in his guest role as the obsessive-compulsive Dr. Kevin Casey]. I could see his Parkinson's disease, and that made me curious to learn more about the Foundation. I checked out the Web site. At that time the home page featured the "Don't Sit Still" campaign, which reflected the humor and optimism Michael and the Foundation bring to their work, while never underestimating the challenges. It really stuck with me.

After I won, Michael called to congratulate me. At first, I couldn't believe it. But we had a really lovely conversation. He promised the Foundation would use the contribution wisely, with great respect and gratitude. That meant the world to me.

MJFF: How did you become interested in food and cooking?

Chris: My family really inspired me to become the chef I am today. I was privileged to always share wonderful food with my relatives, especially my great-grandmother (my uncle's mother), Rosalie, who was from Naples. I gravitated toward kitchens early on — my first job at age 14 was as a dishwasher at IHOP.

I attended culinary school at Johnson & Wales University, and moved to San Francisco in 1996. In addition to my work with Incanto for the past 10 years, I was a co-creator of Bocalone artisanal salumeria, and last March, I opened PIGG, my tribute to all things pork, at UMAMCatessen in Los Angeles. I'm proud of helping popularize the "nose-to-tail" movement among chefs, which elevates cooking with offal — the parts of an animal used for food that aren't skeletal muscle. I like to celebrate the whole animal, and I love to surprise people by taking something they consider not good and making it amazing. [Learn more about Chris's work at his Web site, www.offalgood.com.]

MJFF: You won \$141,000 for the Foundation. Did you know that you actually won twice that much? Every dollar will be doubled by our \$50-million Brin Wojcicki Challenge.

Chris: I didn't know during the competition, so that is pretty cool! I went to Las Vegas to get a job done, and to do it for the right reasons. Win or lose, I could still look myself in the mirror. But it feels so great to win for such an amazing cause.



From left to right, Tom, Claudia, Nikki and Francesca Marshall

When Nikki Marshall of Westport, Connecticut, first got involved with The Michael J. Fox Foundation, she was searching for ways to help her husband, Tom. He'd been diagnosed with Parkinson's in 1994. What she didn't expect was how much her engagement with the Foundation would help her — and their two daughters.

With a daily three-hour roundtrip commute from Westport to New York City, where Nikki is a senior vice president in wealth management at Neuberger Berman, she didn't have much time to spare. But she wanted to meet and interact with other caregivers. Younger than many in her situation, she hadn't found the right fit through caregiver support groups in her area.

As she got to know the Foundation better, she was connected with several Westport women who shared her experiences — including Alison Wachstein, featured on page 6. Just over a year ago, they created an informal group that gets together each month. "We've deliberately kept it intimate, with fewer than 12 of us. These women have made such a difference in my life. Tom and my girls worry about me less, knowing that I'm not alone," Nikki says.

They also keep each other informed. "It was through the group I learned Neupro® [a dopamine agonist patch] was back on the market. After discussing this with his doctor, Tom is now clearly benefitting from the patch," she says.

Her daughters, Francesca and Claudia, have gotten involved, too. Francesca joined the Team Fox Young Professionals of New York, known as the YPs. She's appreciated meeting people her age who also have a parent with PD. In 2010, Francesca ran the ING New York City Marathon with Team Fox, raising almost \$5,000. Nikki recalls attending the pre-race

dinner. "Michael J. Fox took the time to meet every runner. He made them feel so good about what they were doing, and what it meant to him," she says. "It was a very special experience."

Claudia gained a first-hand perspective on the Foundation through a summer internship on MJFF's Research Partnerships team in 2011 and in 2012. Now a senior at Bucknell University, Claudia had "her eyes opened by this experience," says Nikki. "It was very hands-on. She was taken seriously — presenting to the scientists and interacting with the Foundation's senior leadership." Last summer, Claudia won a grant from Bucknell, which they award to students pursuing nonprofit internships.

Inspired by their belief that "if anyone can find a cure, it's the Fox Foundation," Nikki and Tom have supported MJFF financially since 2010. When they learned of the Brin Wojcicki Challenge last year, they wanted to stretch their dollars — and more than doubled their previous gift. Through the Challenge, that increase was matched dollar for dollar.

"We were moved that someone would put that kind of extraordinary commitment behind the Foundation," she says. Nikki hopes the Challenge will ignite others to contribute. "We're all after a cure. This is an opportunity to leverage our impact and to participate in something that's groundbreaking."

Nikki acknowledges it's been a tough road for Tom since his diagnosis. "But he soldiers on and we are so proud of him," she says. And he takes great pride in the action their family has taken through MJFF.

"Growing up, I think the girls felt unable to help their father, who's had Parkinson's for much of their lives. But through the Foundation they've found something they *can* do. It's given all of us an extended community, more hope and the gift of feeling truly inspired," she says.

GET INVOLVED

... IN CLINICAL RESEARCH

Her Clinical Research Participation Gives One Patient New Reasons for Hope

“We will only get new drugs if people volunteer,” says Betsy Barber, of Tampa, Florida. That’s among the many reasons she has participated in eight clinical trials since her Parkinson’s diagnosis 12 years ago.



Betsy Barber

In fact, she connected with her first trial before she even knew she had Parkinson’s disease. She saw an ad in her local paper for a trial at the University of South Florida, looking for individuals with symptoms like hers. During the initial screening with Dr. Robert Hauser, he diagnosed her with PD, which confirmed her growing suspicion. When he explained the research currently taking place, Betsy, a trauma nurse, was eager to take action. And she was soon enrolled in the trial.

Although she works in the medical field, Betsy says she initially didn’t know much about PD. Thanks to her participation in research, she’s gained significant knowledge about the disease. Betsy also appreciates early access to potential therapies. As part of her first trial, she began taking Requip® XL, which she still uses today. Looking toward the future, she’s optimistic about new treatments; if not during her lifetime, she hopes her children and grandchildren might benefit.

Living with PD for over a decade, Betsy continues to work full-time and has adjusted her schedule to best suit her “on”

periods. “I’ve been very fortunate to have a supportive boss and wonderful colleagues,” she says.

Over the years, Betsy has become an advocate for Parkinson’s — within the patient and medical communities. Traveling to hospitals and assisted living homes in her area, she educates the medical staff about PD. She also presents to local support groups, sharing the state of health care and steps patients can take — especially participating in research.

Last year, Betsy was among the first to create a profile on Fox Trial Finder (foxtrialfinder.org), The Michael J. Fox Foundation’s clinical trial matching tool. She says, “There’s a lot of information on the Internet about different clinical trials. It’s hard to know which trial might be right for you. What’s great about Fox Trial Finder is that it does the work for you.” Currently enrolled in a trial, she looks forward to using Fox Trial Finder to connect with the next one.

“While the Parkinson’s experience is different for everyone, it’s still possible to lead a normal life,” Betsy says. “Don’t let the disease define you.”

Taking on Parkinson’s Together through PPMI

Like many couples, in their 39 years of marriage, Alison and Bob Wachstein of Weston, Connecticut, have done so much together. But few can share this: their participation in the Parkinson’s Progression Markers Initiative (PPMI), MJFF’s landmark biomarkers study. This study aims to identify biomarkers for PD, which would be a vital tool in the development of new therapies for patients.

For Alison, PPMI was her first experience as a clinical research participant. For Bob, who has Parkinson’s, it marked his fifth clinical study. The Wachsteins learned about PPMI through the Institute for Neurodegenerative Disorders (IND) in New Haven. There in 2009, Bob was diagnosed and quickly connected with several clinical research opportunities. He jokes, “I’ve willed my body to science.” Along the way, he has learned a lot. “These studies have helped me better understand and deal with my illness,” he says. “And the staff at IND is terrific.”

The couple enrolled in PPMI just over a year ago, Bob first as a newly diagnosed patient and Alison shortly after as a control volunteer. She says, “If I was driving with Bobby to IND to participate, why shouldn’t I do it, too? For me, there was no down side to volunteering. I want to contribute to science in any way I can. Now we can say we’ve had ‘his and hers’ LPs [lumbar punctures].”

Throughout their participation in PPMI, the Wachsteins have been very impressed — with the study and its sponsor, The Michael J. Fox Foundation, as well as its 12 industry partners. “I’m excited the data is already being shared with scientists around the world,” Alison says. “You have a real sense you’re a part of something that’s having an immediate impact.” Their involvement has also given her a sense of hope for improved treatments on the horizon, which could make a difference in her husband’s life.



Alison and Bob Wachstein, with their daughter, Liz

Eager to continue their contributions to science, the Wachsteins have registered for Fox Trial Finder (foxtrialfinder.org) to connect with future studies. They’ve also been inspired to contribute financially to the Foundation, especially while the Brin Wojcicki Challenge is in effect. Last year, they gave \$1,000. The Challenge matched that \$500 increase over their gift in 2010, turning their \$1,000 gift into a \$1,500 gift to MJFF.

Further leveraging the Challenge, in 2012 the couple joined Team Fox, the Foundation’s grassroots fundraising arm. Alison, a portrait photographer, hosted “Foxy Headshots” in September,

in which people received a photography session for a donation to MJFF. They raised nearly \$7,000, which will be doubled by the Challenge.

“We’re happy to do more,” says Bob. “With the Fox Foundation, no matter how you contribute, you know it’s being put to good use right away.” They are actively engaged in the PD community and have their next fundraising event already in the works. For their holiday card, they will share news of the Challenge with their friends and family, and ask for a donation. Alison says, “Taking advantage of the Challenge is simple. We want to do whatever we can to get others on board to help speed a cure.”



Wendy and Rick Tigner:

Defining Parkinson's in Their Lives by Taking Action

Throughout her life, Wendy Tigner of Santa Rosa, California, has loved tennis. Five years ago, she was diagnosed with young-onset Parkinson's disease (YOPD) at age 46. One of her first questions to her neurologist was, "Do you know anyone else like me?"

In fact, he did. He connected Wendy with Alyssa Johnson, a runner with YOPD. Since her diagnosis, Alyssa had continued to run, completing several marathons for Team Fox.

The two women became friends and soon started "Gals with YOPD," a private Facebook group for women like them, whose membership now spans the country. Wendy says, "This group has been so helpful for me, especially as it took me a while to share my PD with others. We can chat about our experiences online and have even met in person."

While close friends and family knew of Wendy's Parkinson's early on, it was several years before she was ready to go public. She recalls advice Michael J. Fox once gave: "He said, 'Do it in your own time. You define PD in your life.' I've tried to let that philosophy guide me," she says.

When Wendy was ready, she shared her diagnosis with millions — on primetime TV. Her husband, Rick Tigner, is the president of Kendall-Jackson Wine Estates. In January 2012, he was featured in the reality TV show "Undercover Boss." During the episode, both Rick and Wendy talk openly about her PD.

Rick says, "I was nervous every day of the filming. But Wendy really knocked it out of the park." Wendy says the greater challenge came after the show aired. "People were worried and would ask how I was. While I appreciate their concern, I don't view PD as an end. And I'm not letting it bring me down."



Richard, Wendy, Lisa and Becky Tigner with Michael J. Fox (center)

In 2011, the Tigners began to take action through Team Fox. Inspired by Wendy's love of tennis, they decided to host a tennis tournament and dinner in October. Their initial goal was to raise \$10,000 for The Michael J. Fox Foundation. Knowing the Brin Wojcicki Challenge would match every dollar, they were eager to raise as much as they could. Wendy says, "I was amazed how my community surrounded me. Everyone wanted to help." They sold out the event and raised over \$65,000 for Parkinson's research — doubled to more than \$130,000 by the Challenge.

Already thinking about their next Team Fox event, the Tigners soon found a unique opportunity. In 2011, Rick had taken part in a celebrity golf classic and gala, hosted by the University of California San Francisco Medical Center and benefitting two charities. For 2012, Rick had been asked to help chair the event. He said he would,

but only if The Michael J. Fox Foundation would be one of the charities to benefit from the event.

They agreed, so the Tigners got to work again. Rick was impressed by the collaboration that took place. He says, "It was really one team and one dream, and we hope to do it again next year." The event raised over \$150,000 for MJFF, all of which would be doubled by the Challenge.

Now, they say, "We're figuring out how we can raise \$1 million as a couple." Rick and Wendy appreciate the single-minded focus of MJFF. "We do this to find a cure," Rick says. "And we know any funds we raise for the Foundation will go straight to that purpose."

The Tigners have gotten involved in other members' events, making donations from Kendall-Jackson Wineries. Rick says, "Team Fox is an amazing network of people, and we're grateful to do our part. We only wish we could do more."

The Tigners' three children are involved with Team Fox, too. Their oldest daughter, Lisa, plans to run the ING New York City Marathon in 2013 as a Team Fox member. "When I was first diagnosed," says Wendy, "our kids didn't know much about Parkinson's and that it progresses over time. But now, they really understand the need to support the search for a cure — and that they can do something."

Says MJFF's Co-Founder and Executive Vice Chairman Debi Brooks, "The Tigner family has thrown their energy and resources into helping speed a cure, and they're inspiring others to get involved. The Foundation is truly grateful to have them as a partner in our mission."

Wendy says, "Rick has always been a great public speaker. But through Team Fox, I've learned to share my story with people, too. It's been such an enriching experience for me. No matter what your age or situation, it's important to keep growing and challenging yourself."



Elvis Costello, Tracy Pollan and Michael J. Fox at "A Funny Thing Happened on the Way to Cure Parkinson's". Held on November 10 at New York City's Waldorf-Astoria, the benefit raised \$4.5 million for the Foundation, with performances by Elvis Costello and The Imposters, Michael J. Fox, Louis C.K. and Denis Leary, as well as special appearances by Katie Couric, Bill and Willie Geist, Chris Cosentino, Donny Deutsch and Amanda Peet.

GET INVOLVED



Team Fox Snapshots: Five Events...and the Members Driving Them

Each year, hundreds of Team Fox members channel their creative energies and their passions to host unique fundraisers. MJFF checked in with a few members around the country to see what inspires them and what advice they have for fellow members.

Party Like a Fox

Where: Chicago, Illinois

Who: Gayle Wolski, Co-Chair, Chicago Young Professionals for Team Fox

Why Team Fox: Our dad was diagnosed with Parkinson's over six years ago and is in the final stages of PD. Our family got involved with Team Fox when my brother and sister-in-law ran the Chicago marathon. And I hosted the after-party. The Chicago YPs and our gala event, which my sister-in-law and I co-chaired, grew organically out of that incredible experience. For us, it's all about joining forces to create a contagiously positive impact on our community.

Why a gala fundraiser: It was either go big or go home. We wanted to create an evening where you could nosh on some of Chicago's finest bites, dance and bid on the silent auction. We thought, if we build it, they will come. And they did!

Greatest challenge: Putting in all the time and resources to pull this off — and meet our \$15,000 fundraising goal. But with a big risk comes a big reward! Over 250 people came and we raised more than \$40,000.

Greatest reward: It was amazing to bring so many people together for the first time in Chicago. For many, they had a personal connection to PD, and you could see it in their eyes. It's really a grassroots effort here, and we've barely scratched the surface.

Best advice: Find the right people to help with your event and play to their strengths. Don't hesitate to put your best resources to use. And remember to have fun!

Greatest benefit to joining Team Fox: Team Fox is responsible for bringing together the Chicago YPs. Because we all have a loved one with PD, we're so driven to find a cure. And I know we'll be friends for life.



Gayle Wolski (bottom row, third from the left)

Winter Gala for Parkinson's Disease Research

Where: Norman, Oklahoma

Who: Nicole Jarvis Zimmerer

Why Team Fox: I was diagnosed with young-onset Parkinson's one year ago, at age 39. I immediately wanted to get involved and do something through Team Fox. But there weren't any events near me in Oklahoma. So I decided to create my own.



Why a gala fundraiser: I'd read about Team Fox mentor's Nancy Mulhearn's gala online. I knew an event like this would resonate with my community. I've lived here my whole life and have a lot of connections, so I thought this played well to my resources. I was confident it was something I could pull off.

Greatest challenge: I work full-time as an OB/GYN, and my mom and my office manager are helping me out. With the Brin Wojcicki Challenge in effect for just a few more months, I needed to host my event while I could still double every dollar I raise. I couldn't afford to wait! And I've received so much support from family and friends to make this happen.

Greatest reward: Many people in my area don't know much about PD. So I'm really looking forward educating folks about Parkinson's and Team Fox. And I hope they have fun so they want to come back next year!

Best advice: When you have Parkinson's, Team Fox is one way you can incorporate PD in your life, versus letting it become your life.

Greatest benefit to joining Team Fox: While I've only been a member for a few months, people have come out of the woodwork to support me and this cause. I'm astonished that I've already had to increase my fundraising goal to \$60,000! People now see me as a spokesperson for Parkinson's. On behalf of the more than 15,000 Oklahomans who have PD, I'm honored to do my part.



Pamper Yourself for Parkinson's



From left to right, Arman and Jamie Askari and Sherri and Marc Blaushild

Where: Cleveland, Ohio

Who: Jamie Askari

Why Team Fox: My husband was diagnosed with YOPD several years ago. Right away, we wanted to focus our efforts on helping Team Fox and MJFF.

Why a spa fundraiser: My sister Sherri had been to an event like this. Everyone enjoys pampering themselves. It's even better when you can do it for a great cause.

Greatest challenge: The logistics — specifically coordinating the schedule with our spa service providers and our guests. Next year, you'll make appointments for services as you arrive, instead of booking in advance.

Best advice: Follow your passion. Let that drive you and your event, even when it gets stressful. Stay focused on the big picture: You're raising money to find a cure for this disease.

Greatest reward: Knowing you pulled the event off! Last year, we raised over \$60,000 for MJFF.

Greatest benefit to joining Team Fox: Being at the MVP Awards Dinner last year. My husband and oldest daughter were there. It was one of the most special nights for us as a family. We are so fortunate to have Team Fox in our life — it's 150 percent the right place for us.

Pasta for Parkinson's

Where: Carbondale, Colorado

Who: Olivia Savard

Why Team Fox: My step-grandfather had Parkinson's, and he passed away last year. I wanted to volunteer and help people like him in assisted living homes or hospitals, but they said I was too young [13 at the time]. There's no age limit with Team Fox!

Why a pasta fundraiser: I've hosted several Pancakes for Parkinson's events for my birthday. But this year, we wanted to make it an after-school event. So we're hosting a pasta party for my 16th birthday.

Greatest challenge: It always comes down to those last few weeks before the event, which are most stressful. But my mom helps me balance everything while I'm in school.

Greatest reward: Meeting new people at each event and hearing their stories. Even when you're in a small town, you learn you're not alone.

Best advice: If people have fun at your event, they'll remember and want to come back next year. So keep building your event and make it an annual thing.

Greatest benefit to joining Team Fox: I used to feel helpless, watching as my step-grandfather's Parkinson's progressed. But now I know I'm doing something to help stop this disease. And I'm putting my town on the map. That's why I'm aiming to raise \$100 for every person in Redstone. With 97 of us, that would mean \$9,700. And all of that would be doubled by the Challenge!



Olivia Savard (third from the left)

Charity Cupcake Challenge

Where: Vero Beach, Florida

Who: Janie Graves Hoover

Why Team Fox: My mom, Jeane, had Parkinson's for nine years, and my sister and I wanted to do something for her. In 2010, we decided to run the ING NYC Marathon for Team Fox. Our mom passed away later that year. Since then, our events have been our tribute to her.

Why a cupcake fundraiser: My sister, Judy, and I loved the TV show "Cupcake Wars." We wanted to try to make that concept into a fundraiser. To keep our event accessible, we ask guests to contribute \$10 each. Every amateur and professional baker brings 60 cupcakes. Supporters taste all the cupcakes and vote 10 times for their favorites. Plus they can buy more votes. This year's overall winner was a pumpkin streusel cupcake (pictured left).



Greatest challenge: Trying to guess how many will attend. And the 10 minutes before the doors open, when you wonder if anyone will come.

Greatest reward: My mom touched so many in her life. It's nice to provide people with a way to do something in return

for her — even now. People have such heart for the cause. Every year someone says, "Your mom would have loved this."

Best advice: Don't procrastinate on the details!

Greatest benefit to joining Team Fox: The Team Fox community. Parkinson's manifests itself in so many ways. It's been so meaningful to connect with people who really understand the impact of PD on your family's life.

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