

## PRIORITIZING PARKINSON'S RESEARCH

Dave Iverson of KQED discusses MJFF's strategy for prioritizing PD research areas with Todd Sherer, the Foundation's Vice President of Research Programs.

A transcript of the interview follows. To listen to a podcast version, visit [http://www.michaeljfox.org/newsEvents\\_podcasts.cfm](http://www.michaeljfox.org/newsEvents_podcasts.cfm)

**Opening Narration:** This is Dave Iverson and welcome to our continuing series of reports on the latest developments in Parkinson's research. Parkinson's disease is a complicated condition and researchers have no shortage of targets to tackle. That means that scientists put forward thousands of proposals each year seeking support for a wide range of research possibilities. So how does an organization like The Michael J. Fox Foundation go about deciding which projects have the most promise? The Foundation's Vice President of Research Todd Sherer says the one thing you don't want to do is sit around and wait until all the answers become clear.

**Todd Sherer:** Sometimes in science there will be a lot of let's wait and figure it out. So rather than sit around and wait for the field, what we really wanted to do is take a more proactive approach. We want to push the envelope. So that makes it riskier but we also think that we can get results faster that way.

**Dave Iverson:** Sherer and his staff want to push the envelope in three high priority research areas. One is to find ways to slow down the progression of Parkinson's. A second is to find new therapies for difficult to treat complications like dyskinesia. And the third is to foster new tools and techniques that would speed the overall pace of research. In this report we'll take a look at each priority area starting with slowing down disease progression. Right now some Parkinson's drugs do a good job of helping with some disease symptoms, like tremor and muscle rigidity, but they don't actually slow down the course of the disease. Todd Sherer says one approach that might inhibit disease progression is called trophic factors.

**Todd Sherer:** Trophic factors, to make an analogy, are fertilizers for the brain. So if you think about a brain that is getting sick, if you could put these factors on, it would make them healthy, it would make the brain cells perform better, stay around longer, interact with the other cells better.

**Dave Iverson:** So in other words, to some extent, it can help you hold on to what you've got and nurture what you've got so that if you're not replacing them, you're at least holding on to the supply that's already inside you?

**Todd Sherer:** Yeah, so the idea behind them is that they could keep what's there from progressing further and then even make them perform better than they were prior to that factor, so it may be neuroprotective in that it prevents further degeneration and it may actually be neurorestorative in that it makes the remaining cells actually perform better as well

**Dave Iverson:** So far clinical trials using trophic factors have yielded mixed results, but Sherer and leading Parkinson's researchers think this approach has lots of potential to slow down Parkinson's disease. Another avenue that might do that is through genetics. Researchers used to think that Parkinson's didn't have a genetics connection but now we know that gene mutations can create the same problems in the brain as standard Parkinson's disease. Mutations in one gene in particular – called LRRK2 – are now believed to cause as much as 2 percent of all Parkinson's cases. And because screening can identify potential genetic cases early on, researchers now have an early window into the disease process.

**Todd Sherer:** So this gives the opportunity to try to study these individuals with the mutation and also their family members who do not have Parkinson's disease but who may carry the mutation, who may be at a risk for developing the disease. So you might be able to uncover some very early changes that may predate the diagnosis of Parkinson's. And the ultimate goal would be if we had a therapeutic, you could start treating people before Parkinson's symptoms become prevalent or seen in those individuals.

**Dave Iverson:** But while these efforts to modify disease progression are promising it's also an enterprise that will take time. That brings us to another foundation research priority – what could help patients right now? One immediate problem for many patients is dyskinesias, excessive, involuntary movements that plague many Parkinson's patients and are actually a side effect of medication.

**Todd Sherer:** Dyskinesia is a major focus for us because it is a current side effect for the current treatment of the disease. Dyskinesias are actually increased movements that can happen in response to taking dopamine based medications. So what happens for a patient over time as they take these medications, they can get a symptomatic benefit of the dopamine drug but then there is a double edged sword, they also get this side effect of increased dyskinesias. So right now the way that's really treated is by reducing the amount of medication the patient gets. So you can reduce the side effect but then you're also reducing the symptomatic benefit. So we've really focused on dyskinesia and I think it could have a pretty significant impact for people with the disease today. A lot of things being developed for dyskinesia right now that are in late stage development. I'm very optimistic in this area.

**Dave Iverson:** The third primary focus for the Fox Foundation is to change the research landscape itself by providing scientists with new tools and techniques, like finding a better way to diagnose and monitor the disease, what's called a biomarker, a topic you can learn more about in another one of our podcasts. Another way the foundation is trying to speed research progress is to foster more collaboration between scientists.

**Todd Sherer:** It's a major problem for scientific research because in one case, what you want is a highly, highly competitive environment because that helps to motivate people and it helps people to work faster and with more vigor and enthusiasm, so you want to have some level of

competitiveness. But what you don't want to have is duplication and waste and people not learning from the mistakes of others and leveraging the work of each other. So what we're trying to do for Parkinson's disease is particularly to get scientists to work together to compliment their expertise, to move projects forward. So we have designed a number of programs to really encourage and require that people bring these teams together to tackle specific problems in Parkinson's, because it's really having people look at it from different directions is how we are going to make progress.

**Dave Iverson:** Similarly, the Foundation also wants to make life easier for scientists by developing critical research tools like laboratory models of how Parkinson's works in people. Improved disease models give researchers a better opportunity to study the condition, making Parkinson's research an attractive destination for scientists.

**Todd Sherer:** If you think about if you're a scientist starting out a new lab. Either it's a biotech company or an academic scientist, you are looking across the field of science, what should you study? What should you work in? And what you would be attracted to is an area of inquiry that has a lot of good models that you could not spend a lot of time trying to recreate the model but you could spend a lot of time studying the disease. So that's really one of our goals here. If you provide these research tools to scientists they can then use their creativity to make new discoveries.

**Dave Iverson:** The goal is to do everything possible to speed research progress and in so doing make life better for people with Parkinson's right now and in the long run. For more information about The Michael J. Fox Foundation visit [michaeljfox.org](http://michaeljfox.org). I'm Dave Iverson.