Michael J. Fox: This is Michael J. Fox. Thanks for listening to this podcast. Learn more about The Michael J. Fox Foundation’s work and how you can help speed a cure at MichaelJFox.org.

Dave Iverson: This is Dave Iverson. There are many challenges to advancing new treatments for Parkinson's, including developing the right research tools and finding enough patients to participate in the clinical trials that test those new treatments. The Michael J. Fox Foundation has been active in both arenas. The Foundation's Fox Trial Finder has helped thousands of people identify and participate in clinical trials. Foundation funding has helped create tools to better measure Parkinson's symptoms and the impact of potential therapies. Both of those activities help pave the way for the development of a new medicine called Gocovri to treat dyskinesia symptoms. It was recently approved by the Food and Drug Administration.

Dr. Rajiv Patni: It’s the first medicine approved by the FDA specifically for the treatment of dyskinesia in person's with Parkinson's disease.

Dave Iverson: Dr. Rajiv Patni is the chief medical officer at Adamas, the pharmaceutical company that developed the drug. Dyskinesias are those uncontrollable movements that are associated with disease progression and long term use of Carbidopa/Levodopa, the standard drug treatment for Parkinson's.

In late August, the FDA approved Gocovri, which is an extended release version of the drug compound Amantadine. An immediate release version of it Amantadine was already being used successfully by some patients on an off-label or unofficial basis. Securing an FDA approved drug for dyskinesia was a long time coming. Dr. Christopher Getz is a neurologist at Chicago's Rush University Medical Center who became frustrated a number of years ago about the lack of approved treatments for dyskinesia.

Dr. Getz: I observed that there was nothing on the market and no drugs in the pipeline for dyskinesia. In the middle portion of my career and when I spoke with company representatives who were in the research and development division of various companies, I got the feedback that there wasn’t a good measure, so why should they go into that area?

Dyskinesia is a highly varied condition that can flare up at different times for different patients with different levels of severity. Without a good way to measure that variability or monitor the impact of potential treatments, drug companies essentially took a pass.

It was really the fact that we needed to go forward with developing a good tool in order to attract companies to come forward and think about their products...
on the shelf and how they could utilized and get into the field of dyskinesia and Parkinson's disease.

Dave Iverson: So Dr. Getz and his colleagues set about trying to develop a better way to measure dyskinesia and its impact on patients. The idea was develop a rating scale that would include physician observations, but as important as the patient's perceptions.

Dr. Getz: We want to know what the clinician sees, but we also want to know the impact of those various aspects of dyskinesia on the patient who's living with it. And that impact is very, very difficult for the clinician to appreciate with his or her scores because dyskinesia is a changing phenomenon. What the doctor may see in a 10 minute investigation or rating is completely different than the patient who is living with this over 24 hours.

Dave Iverson: As Dr. Getz observes, physician and patient perspectives on the severity of a problem can differ. Besides the example of someone whose dyskinesia causes a drink to spill, the physicians developing the scale assumed that would be seen as a real problem. But as they discovered, that wasn't necessarily the case.

Dr. Getz: It really was getting at how was this impacting the patient in terms of the goal as opposed to what actually happened. If there was spilled, but it didn't impact the patient, then it's rated as a lower level where the doctors would have expected that, oh, well, well, if they're spilling water, then automatically that impacts the patient. It's a different view. But an important different because we want the patient's perception of how dyskinesia is affecting activities of daily living.

Dave Iverson: But getting funding support to create a rating scale is not necessarily viewed as a high priority research activity.

Dr. Getz: It is difficult to attract funding sources in the area of clinical assessments. It's as if it's not exciting enough. It's so fundamental, but if you don't have it, you really are blocking the field.

Dave Iverson: But with support from The Michael J. Fox Foundation and the Movement Disorder Society or MDS, Dr. Getz and his colleagues at Rush University Medical Center were able to develop what's now known as the unified dyskinesia rating scale. A series of studies has demonstrated that the new scale did a better job of assessing dyskinesia and the efficacy of possible treatments than earlier attempts to do so. That in turn encouraged pharmaceutical companies like Dr. Patni's, to pursue developing and approved dyskinesia treatment.

Dr. Rajiv Patni: As drug developers trying to develop medicine, which hopefully are providing a meaningful benefit to patients with a particular condition. The mandate is drug X has to make a person feel better or function better or live longer. We often hear this mantra feel, function, live. And so a drug has got to do one of those
three things. The beauty of the unified dyskinesia rating scale, it is a measure of feeling and functioning, so it's very salient in pointing to an example of a scale that is a validated measure for both feeling and functioning. That's why as a company and as an industry, we were so drawn to it.

Dave Iverson: And Dr. Patni adds, having that better measure of feeling and function is critical when it comes to determining if a new drug is working.

Dr. Rajiv Patni: In a clinical trial setting, you need to have a measure that is reliable and interpretable, and has the ability to pick up change to be able to assess whether or not in the trial population the drug is doing what we think it is doing. That is why the investment that The Michael J. Fox Foundation did in the scale. It's critical.

Dave Iverson: And just as critically, the drug's effectiveness had to be measured in a sufficient number of clinical trial participants. That's where the foundations' clinical trial matching tool, Fox Trial Finder, played a key role. Adamas Pharmaceutical was one of the first to use that service, which lets interested research volunteers find the studies that need them.

Put these developments together and it's a reminder that seemingly mundane tasks like developing support tools, such as improved measurement scales and better participant recruiting techniques could play a key role in the development of new treatments for Parkinson's disease. Dr. Getz puts it succinctly.

Dr. Getz: Sexy or not, it had a profound influence on the field.

Dave Iverson: To learn more about dyskinesia, Fox Trial Finder, and the newly approved dyskinesia drug, Gocovri, visit MichaelJFox.org. I'm Dave Iverson.