



Issue 16

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About PPMI

The Parkinson's Progression Markers Initiative (PPMI) is a landmark, international clinical study that aims to find reliable and consistent biomarkers of Parkinson's disease (PD) progression. The study is testing today's most promising biomarker candidates through neuroimaging; the collection of blood, urine, and spinal fluid; and clinical and behavioral tests.

Valid measures of PD could allow scientists to predict, objectively diagnose and monitor disease, as well as determine which medications work and which will not.

PPMI is sponsored by The Michael J. Fox Foundation for Parkinson's Research and funded by a consortium of industry partners and individual donors.

Findings and New Initiatives Shared at the Annual PPMI Meeting

More than 200 coordinators, investigators, industry partners and patients gathered in New York City in early May for the annual PPMI meeting to discuss findings, challenges and future strategy.

"Our study has enormous resources that are widely used," said principal investigator Ken Marek, MD. "We want to continue that strategy moving forward and take advantage of new advancements, technologies and capabilities that can enhance the cohort."

While noting the vast impact of the study, attendees also acknowledged that PPMI asks a lot of its participants. The retention rate of 86 percent is high for such a long, intensive study, but engagement should be fostered. Participation fatigue, including among caregivers, as well as advancing age and disease could jeopardize continued involvement. The study is looking at different data collection methods and appreciation efforts to continue engaging participants.

Linda from Alaska has been in PPMI for over six years and attended the meeting with the PPMI Patient Advisory Committee. "It's truly an honor to participate. Seeing the interaction among colleagues is a rare opportunity. In return, I hope I am able to provide insight from the patient perspective. Witnessing this enthusiasm of all involved gives us hope."

Here we share some of the findings and new initiatives shared at the 2019 PPMI Annual Meeting.

Scientific Findings

PPMI is producing greater understanding of Parkinson's, leading to new ways to define, measure and treat the disease.

Genetics: Analysis of data from PPMI and other studies has identified 90 places in genes linked to Parkinson's risk. With some, researchers can create a genetic risk score based on how many mutations one carries and the strength of the mutations' link to Parkinson's disease (PD).

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Tapping into “Liquid Gold”

Progress. New treatments. That’s what Patti wants.

“What if the cure was sitting out there and donating spinal fluid would really advance the research? I want to help,” says Patti, 62, from Elyria, Ohio.

Patti was diagnosed with Parkinson’s disease in 2011 and joined PPMI at the Cleveland Clinic in early 2012.

“We need to get involved to help find a cure. We’re the ones who can give the samples,” she says of her motivation to enroll in PPMI.

Providing biological samples, such as spinal fluid, is easier said than done. But that doesn’t deter Patti.

She tells herself, “It’s a pinch. It’s a second long,” of the lumbar puncture. She’s developed breathing exercises and when she feels a headache coming on, she lies down and waits for it to pass.

“Truthfully, when I think about the little time of discomfort and what it’s doing for research, it’s phenomenal,” she says.

Patti and all PPMI participants have helped build the most robust collection of Parkinson’s data and biosamples of all time. Right now, experts worldwide are combing PPMI data for clues that could point to new disease measures and therapies.

“They can’t just get biosamples from anybody; they need them from Parkinson’s patients,” says Patti. “As my doctor says, ‘It’s liquid gold.’”



Patti and her husband visit Lake Erie with Cleveland in the background. She participates in PPMI at the Cleveland Clinic.

Easing Pain and Anxiety of a Lumbar Puncture

While a lumbar puncture collects valuable spinal fluid samples that help scientists learn more about Parkinson’s onset and progression, the procedure can understandably elicit some anxiety. Dr. David Walega, Chief of Pain Medicine at Northwestern University who sees many PPMI participants, shared some strategies for making a lumbar puncture more manageable.

1. Ask for Communication.

With a procedure happening behind you and out of sight, it can be unnerving not knowing what’s to come. Ask the physician to explain each step of the procedure before she or he begins and then during the lumbar puncture to preview and narrate each step (i.e., “Next I will ... I am now ...”). This communication can help ease nerves.

2. Take Deep Breaths.

If you feel yourself tensing, take slow, deep breaths. Each exhale should be longer than the last one. Deep breathing helps slow your heart rate and reduces the fight-or-flight response.

3. Try a Lidocaine Patch.

If the needle to numb the area before a lumbar puncture makes you cringe, try an over-the-counter lidocaine patch on your lower spine. Sold under brand names Salonpas and Icy Hot, these patches contain a small amount of numbing medication and take a couple hours to bring on the desired effect. Ask your coordinator in advance about using one and if he or she has one that you could apply early in your clinic appointment.

“It’s nice to see a PPMI research participant on my schedule because it’s usually going to be a straightforward procedure,” said Dr. Walega. “I know I speak for all researchers in saying we are very grateful to study volunteers who are sharing their time, experiences and biological samples to help us better understand and treat disease.”

Scientific Findings, New Initiatives and More Data Collection from PPMI

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People with the highest genetic risk scores are about six times as likely to have PD than people in the lowest risk score category.

PPMI scientists also are looking at what genetic mutations work together and how they modify one another. Initial results show changes in the CTSB gene, for example, may have a greater impact on people with a GBA mutation.

“PPMI data is exquisite and far better than any other data in terms of depth and breadth and quality,” said Andrew Singleton, PhD, head of the PPMI Genetics Study Core.

Mood and Thinking: Information on mood and thinking can help understand disease progression. PPMI has found scores on cognitive tests do not decline much in the first five years with disease, and dementia is uncommon. However, depression and apathy increase.

Disease Development: Some people who entered PPMI with Parkinson’s risk have developed the disease. Looking at biology and symptoms in those people can help point to early measures or markers that could lead to better diagnostics or even preventive treatments.

In one way to measure conversion to PD, an investigator notes slowness and one other symptom in a diagnostic questionnaire. With this criteria, 33 at-risk people have developed PD in PPMI (3 with a GBA mutation, 4 with smell loss, 9 with REM sleep behavior disorder, and 17 with a LRRK2 mutation). The study continues to investigate early indicators and diagnosis of disease.

New Initiatives

Some new projects illustrate PPMI’s continued commitment to improvement.

Brain Imaging: A new add-on study utilizing the PPMI infrastructure aims to optimize brain imaging to measure disease. Fifty newly diagnosed patients will receive a DAT scan and a VMAT scan, which is a newer technology. The purpose of the study is to see if there are advantages to one scan. Preliminary results from PPMI suggest that VMAT may be more sensitive at detecting changes seen in PD. The study also will assess results early in disease (6, 12 and 18 months from first scan) to evaluate if these scans could help determine therapeutic impact early in clinical trials of new drugs.

RNA Sequencing: Scientists have sequenced the RNA (gene messengers) of more than 1,500 participants in PPMI.

“PPMI data is exquisite.”

— Andrew Singleton, PhD
Head of the PPMI Genetics Study Core

Profiling gene expression and the impact of different factors (from age to medication use) can help better define disease progression and highlight potential measures and treatment targets.

Additional Data Collection

Since its launch, PPMI has added new ways to gather information on disease experience and biology. If you’d like more information on these programs, please ask your study coordinator.

Digital Devices: More than 220 participants from the United States are streaming data through the Verily Study Watch, and a first volunteer has started using the Roche PD monitoring smartphone application, which will be utilized in Europe. These devices are collecting information on daily life and activities to better measure disease outside the clinic.

Brain Tissue: The PPMI Pathology Core is collecting brain tissue samples from participants after death to measure changes in the brain. Collection plans are in place for nearly 130 participants, and the core has collected samples from five volunteers. They are planning an expansion to European sites.

Direct Communications: PPMI has started an email and mail communication stream to engage participants outside the clinic. The Follow-up of Persons with Neurologic Disorders (FOUND) program has enrolled 1,170 participants and is in place at all U.S. PPMI sites and six outside the country.

In his parting remarks at the meeting, Marek commented on the evolution of the study and challenged its leaders to think bigger.

“What has been so striking is how much energy we have in the study 10 years in, how much we’re talking about projects that are just beginning or emerging,” he said. “A theme we’ve had is how can we take advantage of the data we have and think bigger. This is a real moment and should invigorate us even further.”

Enroll in Fox Insight

Expand your research impact by joining Fox Insight, an online study from The Michael J. Fox



Foundation to help scientists better understand what it's like to live with disease. Open to people with and without Parkinson's, Fox Insight captures important information through online surveys.

Sign up at www.foxinsight.org to begin sharing information on your medical history, lifestyle factors and more. Any English speakers over age 18, with or without Parkinson's, can participate.

Through a collaboration with consumer genetics company 23andMe, Fox Insight participants with Parkinson's in the United States can access the 23andMe Health + Ancestry Service at no cost and add their genetic information to the study.

Make a Plan for Brain Tissue Donation

Donated brain tissue can build on the robust profile of clinical, biological and imaging data you are contributing as a PPMI participant. PPMI has a pathology core for collection, processing and storage of donated brain tissue from recently deceased study volunteers in the United States. We are currently working on a similar program for our European sites.

You and your legally authorized representative — typically your next of kin such as a spouse, adult child, or sibling — will plan for the donation with the study team, and, after analysis, your representative will receive a letter that summarizes the findings from the study of your brain tissue.

Talk to your site coordinator to learn more about how brain tissue helps advance research and how you can plan for such a donation.

Want to stay informed of the latest scientific developments in PPMI? Visit www.PPMI-info.org. This is the portal for the scientific community to learn more about PPMI and to access the data and samples coming out of the study.



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