The moment has arrived: PPMI is officially under way at 166 sites across the United States and Europe with the remaining U.S. and E.U. sites expected to begin recruiting in spring 2011. Enrollment in the landmark, five-year study — the first of its kind focused exclusively on identifying biomarkers for the progression of Parkinson’s disease — is expected to begin soon at another two sites in the U.S. and three in Europe.

Activated study sites have already enrolled several participants (see Enrollment Update, p. 2), and many more have signed consent to take part in the large-scale study. When enrollment is complete, the study will include 400 newly diagnosed Parkinson’s patients, and 200 people who do not have PD.

"We owe a debt of gratitude to the many people who have made a commitment to this groundbreaking study,” said Michael J. Fox. “Thanks to them, we have a chance to speed development of a new generation of therapies with potential to modify the disease process and transform patients’ lives.”

PPMI’s role in developing breakthrough treatments

A biomarker is any measurable substance, process, or characteristic in the body that is associated with the risk, presence, or progression of a disease. Scientists use biomarkers to predict, diagnose, and monitor diseases as well as determine which therapies work, and which ones do not. Currently, there is no validated progression biomarker for Parkinson’s disease, although research has pointed to some strong possibilities.

PPMI is an observational study. Researchers hope to find biomarkers by studying images of participants’ brains; analyzing samples of blood, urine, and spinal fluid; and administering clinical and behavioral tests.

If a biomarker is found, researchers will have a critical tool in the quest to develop drugs with the potential to slow or stop the progression of Parkinson’s disease. Currently available Parkinson’s treatments only alleviate symptoms temporarily.

“With PD biomarkers in hand, we can establish definitive endpoints for clinical trials of disease-modifying Parkinson’s treatments,” said Kenneth L. Marek, MD, Principal Investigator of PPMI. “Endpoints give us concrete ways of determining whether a new therapy is, or is not, slowing the progression of Parkinson’s, as opposed to treating symptoms.”

Continued on page 2
PPMI Enrollment Update

With help from enthusiastic volunteers like you, PPMI clinical sites have hit the ground running since the study’s launch in September 2010.

As of March 1, 2011, 16 of 21 sites have recruited 51 participants. An additional 31 people have signed consent forms and are awaiting screening and baseline tests.

Sites were selected in part for their ability to recruit the needed volunteers on an ambitious timeline. A number of site investigators are using creative tactics to boost enrollment. Many have developed new ways of reaching out to newly diagnosed Parkinson’s patients about the study, and others have focused on colleagues that refer patients to PPMI by underlining the importance of the study.

Enrollment has been bolstered by a surge in media coverage of PPMI, including nearly 50 print and online articles, several television interviews (including Michael J. Fox’s September 30 interview with Sanjay Gupta on CNN), and more than 150 postings on social media websites.

PPMI sites continue to innovate and implement new ideas for reaching newly diagnosed PD and control participants in their communities. One site recently sent an email to neurologists in their area to ask them to refer patients. In return, one of the physicians himself volunteered as a control participant in the study. Sites share stories and tactics like this with each other so that collectively all sites can reach their ambitious recruitment goal.

Have you got a story to tell about how you got involved with PPMI? Share it with the clinical coordinator at your study site and you may be featured in an upcoming edition of PPMI News!

For the most up-to-date information on PPMI recruitment as the study progresses, visit www.PPMI-info.org.

PPMI enrollment status
As of March 1, 2011

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Michael J. Fox Talks Biomarkers with “America’s Doctor,” Sanjay Gupta, MD

When PPMI officially launched in September 2010, Michael J. Fox sat down for a candid conversation on Parkinson’s disease with Dr. Sanjay Gupta, chief medical correspondent, CNN. In this captivating special report, Michael speaks about his experience with Parkinson’s disease, the Foundation’s landmark study to identify PD biomarkers, and the crucial need for greater participation in clinical trials.

The segment is available for on-demand viewing at www.michaeljfox.org/CNN.

PPMI launches

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Investing in biomarkers

MJFF has long championed biomarker discovery and development, with investments of over $28 million since 2002. MJFF is sponsoring PPMI, which is expected to cost $40 million, with funding from the Foundation and a consortium of industry partners and generous donors, including Abbott, GE Healthcare, Genentech, Pfizer Inc., F. Hoffmann-La Roche, Ltd., and MJFF board member Mrs. Lily Safra. Current efforts to develop and market new drugs for central nervous system disorders cost upwards of $1 billion over a period of nine years. Identification of a biomarker for Parkinson’s could dramatically reduce the cost and time to bring a new, disease-modifying treatment to market.

“The Foundation has long believed that investing in critical research tools for drug development could eliminate a significant barrier to testing new Parkinson’s drugs,” said Katie Hood, CEO of The Michael J. Fox Foundation. “We remain committed to an open, collaborative approach that reduces the cost and time of drug development so that we can get vastly improved therapies into patients’ hands sooner.”

Where in the World Is PPMI?

Visit www.PPMI-info.org for information on all 21 clinical sites where PPMI is taking place across the United States and Europe.
Biomarker Basics

They’re the most critical piece of the drug development puzzle that you’ve never heard of. Brush up on biomarkers and why they matter so much that the biggest names in Parkinson’s drug development are joining forces on a five-year, $40-million study to find them.

What is a biomarker?
A biomarker is any measurable substance, process, or characteristic in the body that can be used to objectively track the presence and/or progression of a disease. It could be a molecule found in blood or spinal fluid; a substance or process in the brain that can be seen with advanced brain imaging techniques; or a change in behavior, function, or perceptions (such as sense of smell).

Isn’t a biomarker basically a gene?
A biomarker can be, but is not necessarily, a gene or genetic mutation. (It’s possible to have one or more genetic irregularities linked to PD but never get the disease.) Many other physical and cellular characteristics could be biomarkers if they are measurable and provide an accurate window into the risk, presence, or progression of Parkinson’s disease.

Do we have a biomarker of Parkinson’s?
While no biomarkers for Parkinson’s progression have been confirmed, researchers have amassed several promising candidates, thanks in large part to MJFF investments of nearly $30 million in biomarker discovery. PPMI will serve as a first-of-its-kind testing ground to validate promising leads in a well-characterized, diverse population of people with and without Parkinson’s disease. New leads may also emerge from PPMI data.

How and where will PPMI researchers look for PD biomarkers?
PPMI will focus on identifying three types of biomarkers:

- **Clinical biomarkers** – physicians will use behavioral assessments and physical exams to track and measure cognition (thinking and memory), mood, and heart rate over time.

- **Imaging biomarkers** – scientists use very sensitive brain imaging tools called PET or SPECT and MRI cameras to reveal and track changes in the brain.

- **Biological biomarkers** — pathologists measure and analyze chemicals found in body fluids such as urine, blood, and spinal fluid. Spinal fluid, which bathes the brain, is expected to be a critical source of potential biomarkers.

Where can I learn more?
Check out www.michaeljfox.org/PPMI for in-depth analysis of biomarkers progress and challenges, including detailed FAQs, a personal message from Michael J. Fox, contact information for your nearest clinical site, PPMI in the news, and a podcast with PPMI Principal Investigator Kenneth L. Marek, MD, president and senior scientist of the Institute for Neurodegenerative Disorders in New Haven, Connecticut.

FEATURED FAQ:

What happens to the samples I donate?
The samples collected in PPMI are “de-identified” (stripped of your personal information to ensure your privacy) and sent to a central storage facility. De-identified samples are analyzed for study-relevant characteristics using state-of-the-art lab procedures. The data from this analysis are entered in a database maintained by the PPMI bioinformatics core at the Laboratory of Neuro Imaging (LONI) at the University of California, Los Angeles. Samples are banked in a central repository.

The data and samples are then made available to qualified Parkinson’s researchers around the world to speed the most promising biomarker validation studies.

You can learn about the database for yourself, and understand how your contributions can help drive research progress, at the official research Web site of PPMI, www.PPMI-info.org.

Thank You, PPMI Volunteers

“Because of you, we have the opportunity to conduct vital research that wouldn’t be possible without your participation. Thank you.”

- Michael J. Fox, Founder
  The Michael J. Fox Foundation
PPMI is finally up and running, but what exactly does that mean for those who are donating time and energy to the study?

PPMI is not an interventional trial, so participants will not receive a new drug. Instead, those who take part in PPMI will be asked to give a few things — including samples of blood, urine, and spinal fluid; brain scans; and clinical and behavioral tests. Researchers can mine these items for clues about the progression of Parkinson’s disease.

Looking for biomarkers in a large, diverse group of people with and without Parkinson’s disease gives us the chance to put everything we’ve learned so far about possible biological triggers for PD to the test. PPMI will create the scientific infrastructure necessary for the validation of biomarkers that we suspect play a role in Parkinson’s progression. Even the most promising drug research can grind to a halt without a scientifically sound therapeutic target. Findings from this study may give PD researchers and industry partners the evidence they need to begin developing targeted new therapies.

Sharing the cost of biomarker development with a consortium of industry partners reduces the burden on any individual company. It’s no wonder drug developers are cautious, given that the cost to develop new drugs for central nervous system disorders can exceed $1 billion. And sharing PPMI data with top-flight Parkinson’s researchers from around the world may accelerate the pace of biomarker development.

Perhaps most important, currently available Parkinson’s treatments temporarily alleviate symptoms but do nothing to prevent the disease from advancing. If PPMI uncovers a biomarker, researchers will have a critical tool to use in the quest for disease-modifying therapies that can slow or stop Parkinson’s progression.

Because of you, we have the opportunity to conduct vital research that wouldn’t be possible without your participation. We may soon uncover a biomarker that reveals processes behind Parkinson’s progression. And we may be able to translate this data into the development of targeted therapies that stop the disease in its tracks. Thank you.

—Michael J. Fox, Founder, MJFF
—Katie Hood, CEO, MJFF
—Ken Marek, MD, Principal Investigator, PPMI

Thank You for Being Part of PPMI