# The Parkinson's Progression Markers Initiative: A Prospective Biomarkers Study

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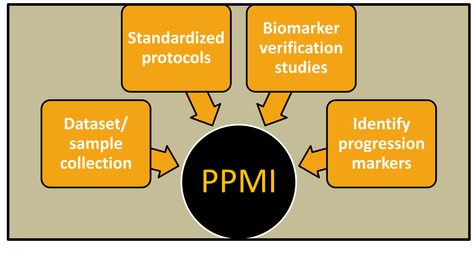
PARKINSON'S PROGRESSION MARKERS INITIATIVE
Play a Part in Parkinson's Research

## BACKGROUND AND RATIONALE

**Current clinical outcomes for Parkinson's disease (PD) trials to assess potential disease modifying therapies require large sample size and long study duration. Reliable and well-validated biomarkers to monitor PD progression would dramatically accelerate research into both PD etiology and therapeutics. During the past two decades much progress has been made in identifying and assessing PD biomarkers, but as yet, no fully validated biomarker for PD is currently available. Given the recent advances in molecular genetics, neurobiology, imaging technology and radiochemistry that have provided new tools that may be useful for PD biomarkers, and the recognition that the lack of PD progression biomarkers has created a roadblock for further studies of disease modifying therapies, there is increasing consensus that a major initiative to develop PD progression biomarkers is both necessary and feasible. The PPMI study is designed to identify clinical, imaging, and biologic biomarkers of Parkinson's disease progression and to standardize the assessment of these tools for future disease modifying trials. The study was launched in June 2010.**

## OBJECTIVES




**Deliverable: Identify a biomarker tool set that can be used to inform decisions at early stages of drug development and clinical testing**

## STUDY CORES

- **Steering Committee:**
  ePI-K Marek, A Siderowf, C Scherzer, D Jennings, K Kieburtz, W Poewe, B Mollenhauer, C Tanner, B Ravina (core leaders, MJFF, ISAB)
- **Clinical Coordination Core:**
  University of Rochester, Karl Kieburtz
- **Imaging Core:**
  Institute for Neurodegenerative Disorders, John Seibyl
- **Statistics Core:**
  University of Iowa, Chris Coffey
- **Bioinformatics Core:**
  Laboratory of Neuroimaging at UCLA, Arthur Toga
- **BioRepository:**
  Coriell/BioRep, Alison Ansbach, Pasquale De Blasio, Michele Piovella
- **Bioanalytics Core:**
  University of Pennsylvania, John Trojanowski, Les Shaw
- **Genetics Core:**
  NIA/NIH, Andy Singleton

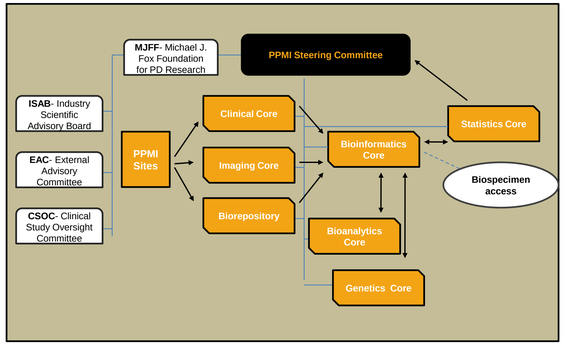
## STUDY DESIGN AND KEY FEATURES

| | |
|---|---|
| **Study population** | •400 *de novo* PD subjects (newly diagnosed and unmedicated)<br>•200 age- and gender-matched healthy controls<br>•Subjects will be followed for a minimum of 3 years and a maximum of 5 years |
| **Assessments/ Clinical data collection** | • Motor assessments<br>• Neuropsychiatric/cognitive testing<br>• Olfaction |
| **Imaging** | •DATscan image at baseline and months 12, 24, and 48<br>•MRI<br>•MRI/DTI at baseline and months 12, 24, and 48 |
| **Biologic collection/ Verification studies** | • DNA collected at screening visit<br>• Blood collected at each visit; CSF collected on an annual basis<br>• Samples aliquoted and stored in central biorepository<br>• Lead biologic candidates potential to be tested: alpha-synuclein, DJ-1, urate |
| **Key Study Features** | • Subject recruitment eligibility includes DAT imaging status<br>•Comprehensive longitudinal biomarker and imaging assessments<br>• Longitudinal CSF acquisition in all study subjects<br>• Standardization of all data acquisition<br>• Flexibility to incorporate novel biomarker candidates<br>• Public-Private partnership in pre-event space<br>• **Data will be available to the PD research community through a web portal**<br>• **Biologic fluids will be available for biomarker verification studies by application** |

## CLINICAL SITES

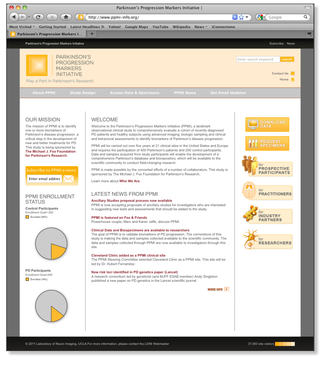
- Banner Research Institute (Phoenix, AZ)
- Baylor College of Medicine (Houston, TX)
- Boston University(Boston, MA)
- Cleveland Clinic (Cleveland, OH)
- Emory University (Atlanta, GA)
- Imperial College of London (London, England)
- Innsbruck University (Innsbruck, Austria)
- Institute for Neurodegenerative Disorders (New Haven, CT)
- Johns Hopkins University (Baltimore, MD)
- Northwestern University (Chicago, IL)
- Oregon Health and Science University (Portland, OR)
- Paracelsus-Elena Klinik (Kassel, Germany)
- The Parkinson's Institute (Sunnyvale, CA)
- University of Alabama at Birmingham (Birmingham, AL)
- University of California San Diego (San Diego, CAO
- University of Napoli (Naples, Italy)
- University of Pennsylvania (Philadelphia, PA)
- University of Rochester (Rochester, NY)
- University of South Florida (Tampa, FL)
- University of Tübingen (Tübingen, Germany)
- University of Washington (Seattle, WA)

## STUDY GOVERNANCE

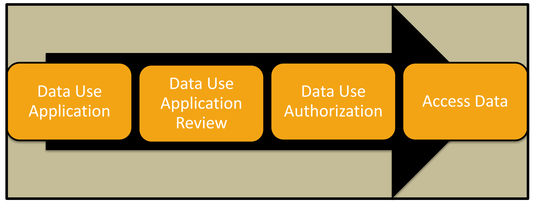


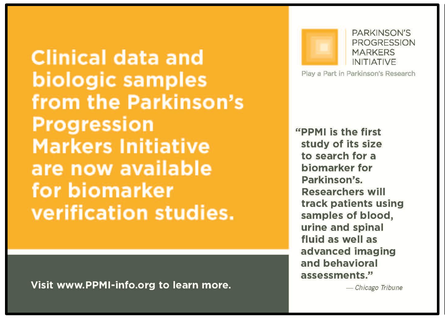

## WEBSITE

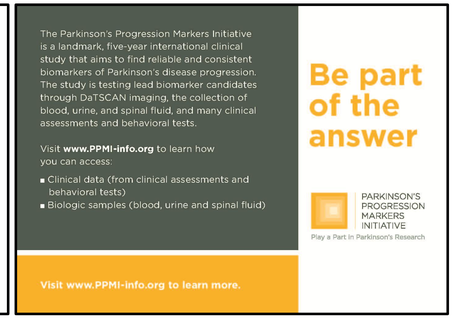
**PPMI study data and biologic samples are available to investigators.**
**For more information, visit: www.PPMI-info.org**



Web-based systems support easy-to-use online application, review, authorization and data access processes.




## STATUS AND RECRUITMENT UPDATE

- First subject recruited - June 2010
- As of May 09, 2011, 107 subjects have been enrolled (64 PD Subjects; 43 Healthy controls).
- Target Date for end of recruitment: Sept 2012

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