

# PPMI Status Update

**Ken Marek**

**PPMI Investigators Meeting  
May 5, 2011  
New York, NY**



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# PD patient vignette

- **67 yo right headed WF in excellent general health**
- **History**
  - 6 month history of poor tennis play**
  - Note 1-2 years – mild constipation**
  - 2 months intermittent R UE tremor while reading the newspaper, or if in stressful situation**
- **Exam**
  - Mild R UE resting tremor**
  - Reduced R arm swing**
- **PD DIAGNOSIS – 1 MONTH AGO**
- **“IF THE SYMPTOMS REMAIN AS THEY ARE NOW – I COULD DEAL WITH THIS”**



# PPMI-PD patient vignette

- **62 yo right headed WM lawyer in excellent general health**
- **History**
  - 9 month history of slowly worsening R UE tremor**
  - 6 month history of R shoulder pain**
  - 3 months voice less reliable in public speaking**
- **Exam**
  - Mild R UE resting tremor**
  - Mild R bradykinesia**
- **PD DIAGNOSIS – 3 MONTH AGO**
- **“PPMI is attractive because no meds and I can do something that will help research”**



# PPMI-Control vignette

- **62 yo right headed WF school principal in excellent general health**
- **History**  
**Husband has PD for 17 years**  
**No previous participation in clinical research**
- **Exam**  
**Normal**
- **“PPMI is something I can do for my husband even if he can’t join the study”**



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# Parkinson's Progression Markers Initiative

## Requirements for Biomarker Infrastructure

### Specific Data Set

- Appropriate population (early stage PD and controls)
- Clinical (motor/non-motor) and imaging data
- Corresponding biologic samples (DNA, blood, CSF)

### Standardization

- Uniform collection of data and samples
- Uniform storage of data and samples
- Strict quality control/quality assurance

### Access/Sharing

- Data available to research community → data mining, hypothesis generation & testing
- Samples available for studies



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# Establish a Specific Data Set

- **Study governance - weekly meetings of the executive steering committee and monthly meetings of the full steering committee consisting of all study cores**
- **All study cores including clinical, imaging, biorepository, bioanalytics, stats, bioinformatics and genetics – established and working**
- **Twenty-one clinical sites (16 US, 5 EU)**
- **Seventeen sites have been trained and activated (14US, 3EU)**
- **All have fully executed contracts**
- **Recruitment of PD subjects and healthy controls**
- **PPMI subjects are successfully undergoing baseline and follow-up assessments including CSF collection**



# PPMI Study

**MJFF**- Michael J. Fox Foundation for PD Research

**PPMI Steering Committee**

**ISAB**- Industry Scientific Advisory Board

**EAC**- External Advisory Committee

**CSOC**- Clinical Study Oversight Committee

**PPMI Sites**

**Clinical Core**

**Imaging Core**

**Biorepository**

**Bioinformatics Core**

**Bioanalytics Core**

**Genetics Core**

**Statistics Core**

**Biospecimen access**

**Data access**





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# PPMI Clinical Sites - 2011

- **University of Rochester – Rochester NY**
- **Oregon Health Sciences University – Portland OR**
- **Baylor College of Medicine – Houston TX**
- **The Parkinson's Institute – Sunnyvale CA**
- **University of Pennsylvania – Philadelphia PA**
- **University of South Florida – Tampa FL**
- **University of California San Diego - San Diego CA**
- **Johns Hopkins University – Baltimore MD**
- **Emory University, School of Medicine – Atlanta GA**
- **Institute for Neurodegenerative Disorders - New Haven CT**
- **Boston University – Boston MA**
- **University of Alabama at Birmingham – Birmingham AL**
- **Northwestern University – Chicago IL**
- **Univ. of Wash & VA Puget Sound Health Care System – Seattle WA**
- **Cleveland Clinic – Cleveland OH**
- **Banner Research Institute- Phoenix AZ**
  
- **Innsbruck Medical University – Innsbruck Austria**
- **University of Napoli – Napoli Italy**
- **University of Tubingen – Tubingen Germany**
- **Paracelsus-Elena Klinik – Kassel Germany**
- **Imperial College of London – London England**



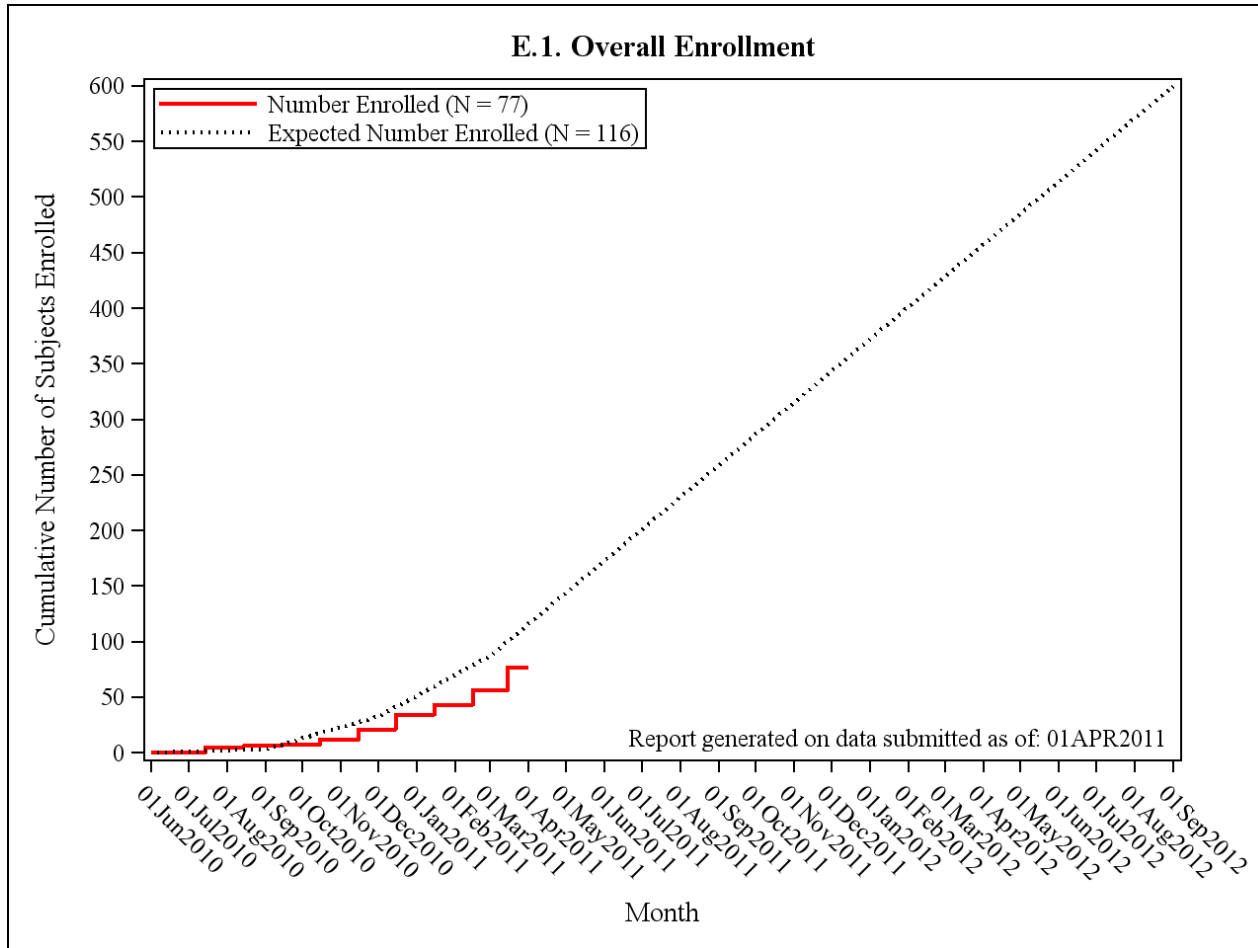
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# ENROLLMENT (through April, 2011)



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# Recruitment – Early lessons learned

- **It is do-able, but challenging to recruit at planned rate – 1 PD/month, 1 control/2months**
- **Multiple strategies to enhance recruitment are necessary**
- **Control recruitment may be easier than PD recruitment**
- **LPs are not a major deterrent for potential subjects**
- **Datscan availability has been a major challenge – DAT imaging as eligibility criteria not fully tested**



# Recruitment – Retention - moving forward

- **Ongoing recruitment will require continued efforts**
- **Retention strategies are crucial**
  - **Continued participation in all assessments**
  - **Need for PD medications**
- **PPMI cumulative subject planning**
  - **Subject assessments**
  - **Subject data**
- **PPMI as an iterative study**
  - **Additional assessments/studies/cohorts**
  - **D/C assessments**



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# Standardization of data acquisition/analysis

- **Manuals/SOPs for all data acquisition**
- **Training for biosample collection and shipping, UPDRS, neuropsych, imaging acquisition and data transfer, clinical data entry.**
- **Quality control of biosamples, imaging data**



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# Data/Biosample Access/Sharing

[www.ppmi-info.org](http://www.ppmi-info.org)

- **Biologic samples available via website**
- **Data available via website**
- **Ancillary study application through website**



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**Bioinformatics Core**

**Bioanalytics Core**

**Genetics Core**

**Statistics Core**

**Biospecimen access**

**Data access**



# PPMI SC and Study Cores

<b>Steering Committee</b>	PI-K Marek, A Siderowf, C Tanner, D Jennings, K Kieburtz, W Poewe, B Mollenhauer, T Simuni, (core leaders, MJFF, ISAB), S Lasch
<b>Clinical Coordination Core</b>	<ul style="list-style-type: none"> <li>▪ University of Rochester's Clinical Trials Coordination Center</li> <li>• PI: Karl Kieburtz, Emily Flagg, Alice Rudolph, Cindy Casaceli</li> </ul>
<b>Imaging Core</b>	<ul style="list-style-type: none"> <li>▪ Institute for Neurodegenerative Disorders</li> <li>• PI: John Seibyl, Norbert Schuff, Susan Mendick</li> </ul>
<b>Statistics Core</b>	<ul style="list-style-type: none"> <li>▪ University of Iowa</li> <li>• PI: Chris Coffey, Qing Yang</li> </ul>
<b>Bioinformatics Core</b>	<ul style="list-style-type: none"> <li>▪ Laboratory of Neuroimaging (LONI) at UCLA</li> <li>• PI: Arthur Toga, Karen Crawford</li> </ul>
<b>BioRepository</b>	<ul style="list-style-type: none"> <li>▪ Coriell/BioRep</li> <li>• PI: Alison Ansbach,</li> <li>• Pasquale De Blasio, Michele Piovella</li> </ul>
<b>Bioanalytics Core</b>	<ul style="list-style-type: none"> <li>▪ University of Pennsylvania</li> <li>• PI: John Trojanowski, Les Shaw</li> </ul>
<b>Genetics Core</b>	<ul style="list-style-type: none"> <li>▪ National Institute on Aging/NIH</li> <li>• PI: Andy Singleton</li> </ul>



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# PPMI Committees

- **Biologics**
    - John Trojanowski
    - Les Shaw
  - **Imaging**
    - John Seibyl
  - **Neuropsych /Neurobehavior**
    - Andrew Siderowf
  - **Sleep**
    - Wolfgang Oertel
  - **Genetics**
    - Andrew Singleton
  - **Biospecimen review**
    - Gene Johnson
  - **Data and publication**
    - David Standaert
  - **Ancillary study**
    - Carlie Tanner
  - **Recruitment and retention**
    - Danna Jennings
  - **Website**
    - Carlie Tanner
- 
- **CSOC**
    - Ron Pfeiffer
  - **External advisory Board**
    - Guy McKhann
  - **Patient Advisory Board**



# PPMI MJFF team

- **Sohini Chowdhury, PPMI Overall Project Manager**
- **Jamie Eberling, PhD, Imaging Core and imaging SOPs**
- **Mark Frasier, PhD, Biologics (Biorepository selection; biologic collection SOPs, assay identification and optimization)**
- **Claire Meunier, Recruitment/Retention Strategies**
- **Debi Brooks, Industry partnership development, Recruitment/Retention Strategies**
- **Todd Sherer, PhD, MJFF VP, Research Programs**



# PPMI Funding Partners

PPMI is sponsored and partially funded by The Michael J. Fox Foundation for Parkinson's Research. Other funding partners include a consortium of industry players, non-profit organizations and private individuals.



# Early lessons

- **We can do this**
- **PPMI will require a large cooperative efforts form all PD constituencies - there is interest worldwide**
- **PPMI must continue to be innovative – not a standard clinical study**



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# Annual Investigators Meeting

## May 5, 2011

### AGENDA

7:30-8:30 am	<b>Breakfast</b>		All		
8:30-8:45 am	<b>Welcome and Introductions</b>		Marek, Sherer		
8:45-9:15 am	<b>PPMI Status Update</b>		Marek		<b>Lunch 12:15 -1:15</b> <b>All</b>
	<ul style="list-style-type: none"> <li>• Sites</li> <li>• Recruitment data</li> <li>• Enrollment</li> <li>• Taskforces, Committees, ISAB</li> <li>• Early lessons learned</li> </ul>				
9:15-9:45 am	<b>Clinical Data Recap</b>		Coffey, Kiebertz	1:15-1:30 pm	<b>Report from Industry Scientific Advisory Board</b> Grachev
	<ul style="list-style-type: none"> <li>• Demographic Information</li> <li>• Collected data (motor, non-motor, neuropsych and neurobehavioral)</li> <li>• Data entry and future training</li> </ul>			1:30-2:10 pm	<b>Assay Preparation</b> Frasier, Shaw, Trojanowski
				2:10-3:10 pm	<b>Recruitment, Enrollment and Retention</b> Jennings, Meunier, All
9:45-10:05 am	<b>Imaging Recap</b>		Seibyl, Schuff		<ul style="list-style-type: none"> <li>• Efforts to date, on-going and future plans and strategies</li> <li>• Recognition of sites excelling at recruitment</li> </ul>
	<ul style="list-style-type: none"> <li>• Update and data on DaTSCAN, DTI and MRI</li> <li>• Future training</li> </ul>			3:10-3:20 pm	<b>Break</b>
10:05-10:20	<b>DAT Imaging Status</b>		Marek	3:20-4:20 pm	<b>Ancillary Studies</b> Siderowf, Tanner
10:20-10:40 am	<b>Break</b>			4:20-4:50 pm	<ul style="list-style-type: none"> <li>• Process</li> <li>• Studies</li> </ul>
10:40-11:10 am	<b>Biologics Recap</b>		Scutti, Frasier Shaw	4:50-5:00 pm	<b>Future Plans</b> Marek
	<ul style="list-style-type: none"> <li>• Update on inventory/process</li> <li>• Data on received samples</li> <li>• Future training</li> <li>• Biospecimen request process/Biospecimen Review Committee (BRC)</li> </ul>				<ul style="list-style-type: none"> <li>• Additional sites worldwide</li> <li>• Pre-motor cohorts</li> <li>• 2012 Annual Meeting dates</li> </ul>
11:10-11:45 am	<b>Planned PPMI Analyses</b>		Coffey, Marek		<b>Closing</b> Marek
11:45-12:10 pm	<b>Data Access Process</b>		Toga, Crawford, Standaert		



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