Ancillary Studies
PPMI Ancillary Study Proposals

• Investigators are invited to propose ancillary sub-studies for PPMI
  – initiate the process through the PPMI web site

• These sub-studies may include
  – Analysis of an existing dataset
  – Additional study assessments
  – May involve all or a subset of PPMI participants
PPMI Ancillary Study Proposals

• Easy access e-Form located on PPMI study website

• Straight forward brief process which allows uploading of an ancillary proposal

• Application process is available 24/7 with submissions sent directly to the committee chair
PPMI Ancillary Study Proposals

• Proposals are accepted on a rolling basis

• Proposals are reviewed on the following criteria
  – The scientific merit of the proposal
  – Value added to PPMI
  – Additional burden to the subject, clinical site and central administration of PPMI
  – Feasibility within the PPMI timeline.
PPMI Ancillary Study Proposal

- **Letter of Intent (LOI) 2 pages and includes**
  - brief description of the proposed sub-study
  - specific goals
  - background and rationale
  - preliminary data to support the proposal
  - proposed additional or modified assessments
  - estimated sample size (including special characteristics of the population)
  - additional resources available and/or required to complete the proposal
  - any potential or available source of funding for the proposal

- **Ancillary Study Committee review of the LOI**
  - Notification via email within two weeks of submitting the LOI
    - Yes/No decision; Critique not provided
  - Investigator may be invited to submit a Full Proposal
  - Investigators must identify funding: no funds through PPMI. May apply through established MJFF grant programs or other sponsors.
PPMI Ancillary Study Proposal

• Full Proposals
  – 5 pages in length
  – Follow the format for MJFF research proposals
    [link: www.michaeljfox.org/research]
  – Reviewed by the PPMI Ancillary Study committee (& subject experts as needed) within 8 weeks of submission date
  – No detailed written critique
PPMI Ancillary Study Proposal

Review Criteria

• Consistent with & furthers the overall PPMI goals of developing biomarkers for the progression, prognosis or diagnosis of PD?

• Sufficient preliminary data to justify using PPMI cohort?

• Does not add undue subject burden or detract from the main PPMI protocol

• Expertise, resources and environment of investigator(s)

• Willing to comply with PPMI policies including Publication and Intellectual property

• Data generated from analyses of PPMI data returned; public access
PPMI Ancillary Study Proposal

Committee members:
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Ken Marek
Todd Sherer
Andrew Siderowf
Tanya Simuni
Carlie Tanner (Chair)
Eduardo Tolosa
Current Ancillary Studies

• Longitudinal follow-up of screen failure due to scan without dopamine deficit (SWEDD)

• Feasibility and reliability of home dexterity testing using the OPDM-dexterity measure
Primary Objective
To evaluate the probability of a change in the clinical diagnosis of PPMI PD subjects with a baseline DaTSCAN that shows no evidence for DAT deficit (SWEDD)

Secondary Objectives
To compare baseline characteristics of SWEDD to non-SWEDD PD subjects and healthy controls
- clinical characteristics
- biomarker characteristics
To evaluate the change in DAT uptake in the SWEDD subjects over a 24-month period and compare it with change in DAT uptake for PPMI PD subjects
To determine the change in clinical markers over a 24-month period compared to PD patients and normal controls
SWEDD follow up

Subjects
Subjects identified as PD patients for PPMI who are excluded based on a normal DatSCAN at screening

Assessments
24 month follow up: visits at 6 months, 12 months, 18 months (phone) and 24 months
  Clinical (motor, non-motor, neuropsychological)
  Biomic blood draw
DaTSCAN at baseline and 24 months
Home dexterity testing study

Primary objective
To assess the feasibility of incorporating home dexterity testing using the OPDM-Dexterity measure into a longitudinal observational study of progression of Parkinson’s disease (PPMI)

Secondary objectives
• To assess the reliability of home dexterity testing over repeated short-term administrations
• To assess the validity of home dexterity testing relative to examiner-based measures (e.g. UPDRS)
• To assess the sensitivity to change of dexterity testing by comparing scores at baseline and year 1.
Home dexterity testing study

Subjects
- 15 PPMI PD subjects at 3 sites (OHSU, INDD, UPenn) total of 45 subjects

Assessments
- Home testing with OPDM dexterity device at least 3 times a month for 3 months
- In person OPDM testing at clinic visits at baseline, 3 months, 6 months and 12 months
- Comparison to UPDRS assessments collected during normal PPMI visits
Current ancillary studies: consistent with review criteria

• Consistent with and furthers the overall goals of PPMI study

• Feasible within parent study
  – Limited additional subject/site burden