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
  - Should take into consideration progress in PPMI
  - Need for funding
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
    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:
Sohini Chowdhuri
Chris Coffey
Danna Jennings
Shirley Lasch
Ken Marek
Todd Sherer
Andrew Siderowf
Tanya Simuni
Carlie Tanner (Chair)
Eduardo Tolosa
Ancillary Studies

Active

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 (TAP-PD)

Assessment of dementia and MCI in PPMI

Proposed

Synuclein and DJ-1 in saliva (under NIH review)

Resting state MRI (implementation imminent)

Linking Clinical Data & Molecular Function in using Patient Specific Stem Cells (Referred to Biologics Working Group)
SWEDD follow up

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 clinically as PD patients for PPMI who do not meet PD eligibility based on a normal DatSCAN at screening

Assessments
24 month follow up (visits at 6 months, 12 months, 18 months and 24 months)
   Clinical (motor, non-motor, neuropsychological)
   Biomic blood draw
DaTSCAN at baseline and 24 months
Baseline Demographics (SWEDDS)

<table>
<thead>
<tr>
<th>Characteristic (n = 25)</th>
<th>Mean</th>
<th>Range; s/d</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age (years)</td>
<td>58.4</td>
<td>38 – 75; 10.9</td>
</tr>
<tr>
<td>Gender (percent male)</td>
<td>52%</td>
<td>N/A</td>
</tr>
<tr>
<td>Symptom duration (months)</td>
<td>9.5</td>
<td>1.0 – 37.0; 9.2</td>
</tr>
<tr>
<td>MDS-UPDRS, part III score</td>
<td>16.2</td>
<td>3 – 39; 9.7</td>
</tr>
<tr>
<td>Tremor (percent present)</td>
<td>84%</td>
<td>N/A</td>
</tr>
<tr>
<td>UPSIT (raw score)</td>
<td>30.8</td>
<td>12 – 39; 6.6</td>
</tr>
<tr>
<td>Number of PPMI sites with at least one SWEDD</td>
<td>11</td>
<td>N/A</td>
</tr>
</tbody>
</table>
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
- Goal: 15 PPMI PD subjects at 3 sites (OHSU, INDD, UPenn) total of 45 subjects
- Current status: OHSU = 3; INDD = 7; UPenn = 4

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
### TAP-PD baseline demographics

<table>
<thead>
<tr>
<th>Characteristic (n=10)</th>
<th>Mean</th>
<th>Range</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age</td>
<td>62</td>
<td>50-81</td>
</tr>
<tr>
<td>Gender (% male)</td>
<td>80%</td>
<td></td>
</tr>
<tr>
<td>OMS</td>
<td>41.2</td>
<td>24.1-48.6</td>
</tr>
<tr>
<td>Key Down</td>
<td>3.9</td>
<td>3.4-4.3</td>
</tr>
<tr>
<td>Peg Cycle</td>
<td>0.9</td>
<td>0.8-1.4</td>
</tr>
</tbody>
</table>
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