PPMI Data Analyses

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Source of data for this presentation:

- All data comes from a data freeze based on data obtained from the LONI website on 04/02/12
Planned Analysis #1: Comparison of Baseline Characteristics Among Health Subjects and PD Subjects.

- Continuous variables assessed using t-test
- Dichotomous variables assessed using chi-square test
- Appropriate assumptions will be assessed for each comparison and any necessary adjustments (i.e., transformations) will be made prior to analysis
Planned Analysis #2: Comparison of Short-Term Change in Progression Endpoints.

- Examine short-term change during first six months for each progression endpoint using mixed model (continuous endpoints) or logistic regression (dichotomous endpoints)

- Initial model will include all baseline characteristics, indicator for whether healthy control of PD patient, and all possible two-way interactions

- Will utilize backwards selection to build a model for each progression endpoint
Planned Analysis #3: Examination of Whether Short-Term Change in Progression Endpoints is Predictive of Change in Long-Term Endpoints

- Consider only progression endpoints that show differences between healthy subjects and PD patients.
- Primary focus on long-term change in UPDRS score – additional long-term endpoints may be considered as well.
- Ten-fold cross-validation procedure will be used to test predictive validity of each model.
- If successful, final model will provide subset of short-term progression endpoints predictive of change in long-term endpoints – suggest biomarkers for future studies of interventions in PD patient populations.
Planned Analysis #4: Examination of PD Subsets

- Each of first three sets of analyses will be repeated comparing subsets of PD patients

- If successful, final model will determine whether some short-term progression endpoints are more predictive of long-term endpoints for some subsets of PD patients and less predictive for other subsets
Planned Analysis #5: Proportion of SWEDD subjects that have a change in diagnosis over 24 month evaluation period

- Percentage and 95% confidence interval will be reported
- Other possible diagnoses will be further divided into 2 categories:
  - Other parkinsonian syndrome with a dopamine transporter deficit
  - Other condition with a dopamine transporter deficit
Planned Analysis #6: Exploratory analysis of SWEDD subjects

- Important changes over time found in planned analyses 1-3 will be assessed in the SWEDD subjects
- Will help to assess whether changes over time in SWEDD subjects are similar or dissimilar to PD subjects
## UPDRS OVER TIME

**Table:**

<table>
<thead>
<tr>
<th>Group</th>
<th>Baseline Median (N) (Min, Max)</th>
<th>Month 3 Median (N) (Min, Max)</th>
<th>Month 6 Median (N) (Min, Max)</th>
<th>Month 9 Median (N) (Min, Max)</th>
<th>Month 12 Median (N) (Min, Max)</th>
</tr>
</thead>
<tbody>
<tr>
<td>PD</td>
<td>32.5 (188) (7, 72)</td>
<td>33 (140) (7, 72)</td>
<td>38 (112) (9, 94)</td>
<td>35 (54) (9, 63)</td>
<td>46 (50) (13, 84)</td>
</tr>
<tr>
<td>HC</td>
<td>3 (147) (0, 20)</td>
<td>N/A</td>
<td>N/A</td>
<td>N/A</td>
<td>4 (27) (1, 14)</td>
</tr>
<tr>
<td>SWEDD</td>
<td>27 (25) (7, 64)</td>
<td>23 (11) (6, 57)</td>
<td>21 (5) (13, 40)</td>
<td>13 (3) (12, 21)</td>
<td>17 (3) (15, 37)</td>
</tr>
</tbody>
</table>

NOTE: Points are only plotted if 5 or more subjects have data at that visit.
## SCOPA-AUT Over Time

<table>
<thead>
<tr>
<th>Group</th>
<th>Baseline Median (N) (Min, Max)</th>
<th>Month 6 Median (N) (Min, Max)</th>
<th>Month 12 Median (N) (Min, Max)</th>
</tr>
</thead>
<tbody>
<tr>
<td>PD</td>
<td>9.0 (188) (0, 39)</td>
<td>10.0 (80) (0, 26)</td>
<td>11.0 (37) (2, 30)</td>
</tr>
<tr>
<td>HC</td>
<td>5.0 (147) (0, 20)</td>
<td>N/A</td>
<td>6.1 (27) (0, 21)</td>
</tr>
<tr>
<td>SWEDD</td>
<td>12.0 (25) (2, 30)</td>
<td>8.0 (5) (6, 20)</td>
<td>9.0 (3) (7, 22)</td>
</tr>
</tbody>
</table>

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### MODSEADL OVER TIME

<table>
<thead>
<tr>
<th>Group</th>
<th>Baseline Median (N) (Min, Max)</th>
<th>Month 3 Median (N) (Min, Max)</th>
<th>Month 6 Median (N) (Min, Max)</th>
<th>Month 9 Median (N) (Min, Max)</th>
<th>Month 12 Median (N) (Min, Max)</th>
</tr>
</thead>
<tbody>
<tr>
<td>PD</td>
<td>90 (188) (80, 100)</td>
<td>90 (143) (75, 100)</td>
<td>90 (106) (70, 100)</td>
<td>90 (55) (70, 100)</td>
<td>90 (38) (70, 100)</td>
</tr>
<tr>
<td>SWEDD</td>
<td>95 (25) (80, 100)</td>
<td>95 (11) (80, 100)</td>
<td>100 (5) (90, 100)</td>
<td>100 (3) (90, 100)</td>
<td>100 (3) (90, 100)</td>
</tr>
</tbody>
</table>

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## GDS OVER TIME

<table>
<thead>
<tr>
<th>Group</th>
<th>Baseline Median (N) (Min, Max)</th>
<th>Month 6 Median (N) (Min, Max)</th>
<th>Month 12 Median (N) (Min, Max)</th>
</tr>
</thead>
<tbody>
<tr>
<td>PD</td>
<td>2 (188) (0, 13)</td>
<td>2 (114) (0, 10)</td>
<td>2 (38) (0, 10)</td>
</tr>
<tr>
<td>HC</td>
<td>1 (147) (0, 15)</td>
<td>N/A</td>
<td>0 (27) (0, 4)</td>
</tr>
<tr>
<td>SWEDD</td>
<td>1 (25) (0, 14)</td>
<td>1 (5) (0, 4)</td>
<td>2 (3) (0, 3)</td>
</tr>
</tbody>
</table>

NOTE: Points are only plotted if 5 or more subjects have data at that visit.
MOCA OVER TIME

<table>
<thead>
<tr>
<th>Group</th>
<th>Baseline Median (N) (Min, Max)</th>
<th>Month 12 Median (N) (Min, Max)</th>
</tr>
</thead>
<tbody>
<tr>
<td>PD</td>
<td>26.95 (190) (20, 30)</td>
<td>26.95 (37) (19, 30)</td>
</tr>
<tr>
<td>HC</td>
<td>28.05 (147) (27, 30)</td>
<td>28.05 (27) (24, 30)</td>
</tr>
<tr>
<td>SWEDD</td>
<td>28.05 (25) (23, 30)</td>
<td>28.05 (3) (24, 28)</td>
</tr>
</tbody>
</table>

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### SDM OVER TIME

**Group** | **Baseline Median (N) (Min, Max)** | **Month 12 Median (N) (Min, Max)**
---|---|---
**PD** | 42.0 (188) (16, 76) | 43.0 (37) (17, 59)
**HC** | 47.0 (147) (20, 83) | 49.0 (27) (27, 79)
**SWEDD** | 43.0 (25) (19, 71) | 50.1 (3) (46, 57)

**SDM over time**

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PLANNED ANALYSES

- Encourage data investigation/mining – know the data
- Propose analyses to working groups, Stats, SC
- What can be done now
  - Correlations of baseline data
  - Study design and assessment question
    - LP
  - Recruitment
  - MDS-UPDRS correlations with non-motor