PPMI Clinical Data Recap

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Source of data for this presentation:

- Information comes from:
  - Tables produced for CSOC report (to be presented during 05/10/11 call)
  - Tables produced for monthly review by steering committee
- All data comes from a data freeze based on data obtained from the LONI website on 04/01/11
PD Subjects:

- 85 consented
  - 50 enrolled
  - 19 pending / 16 declined or excluded

Healthy Controls

- 56 consented
  - 36 enrolled
  - 12 pending / 8 declined or excluded

86 Subjects Enrolled
ENROLLMENT (CSOC REPORT)

PD Subjects:

- 73 consented
  - 46 enrolled
  - 14 pending / 4 declined / 9 excluded

Healthy Controls

- 45 consented
  - 31 enrolled
  - 7 pending / 5 declined / 2 excluded

77 Subjects Enrolled
E.1. Overall Enrollment

Enrollment at approximately 66% of scheduled recruitment

Report generated on data submitted as of: 01APR2011
ENROLLMENT (CSOC REPORT)

E.2. PD Subject Enrollment

Report generated on data submitted as of: 01APR2011

Month

Cumulative Number of Subjects Enrolled

Number Enrolled (N = 46)

Expected Number Enrolled (N = 77)

E.3. Healthy Control Enrollment

Report generated on data submitted as of: 01APR2011

Month

Cumulative Number of Subjects Enrolled

Number Enrolled (N = 31)

Expected Number Enrolled (N = 39)
REASONS FOR EXCLUSION

PD Subjects:

- 9 excluded subjects
  - 7 did not meet inclusion criteria (6 due to DaTSCAN)
  - 1 exclusionary medication
  - 1 uncertain diagnosis

Healthy Controls

- 2 excluded subjects
  - Both did not meet inclusion criteria
    (Low MoCA score & essential tremor)
Of the 77 enrolled subjects:

- 45 (58%) have all baseline information entered into the publicly-accessible database
- 72 (94%) have cognitive testing
- 69 (90%) have a urine sample
- 69 (90%) have a blood sample
- 69 (90%) have a lumbar puncture
- 61 (79%) have all clinical forms entered
- 58 (75%) have an MRI
- 55 (71%) have a DaTSCAN

Lag in baseline data completeness is primarily due to time lag between data appearing in clinical database versus the final LONI database.
**GENDER/AGE DISTRIBUTION**

- **PD Subjects:**

<table>
<thead>
<tr>
<th>Gender</th>
<th>Age</th>
<th>Observed</th>
<th>Expected</th>
</tr>
</thead>
<tbody>
<tr>
<td>Male</td>
<td>&lt;56</td>
<td>5</td>
<td>10.6</td>
</tr>
<tr>
<td>Male</td>
<td>56-65</td>
<td>9</td>
<td>11.0</td>
</tr>
<tr>
<td>Male</td>
<td>&gt;65</td>
<td>14</td>
<td>8.7</td>
</tr>
<tr>
<td>Female</td>
<td>&lt;56</td>
<td>5</td>
<td>5.5</td>
</tr>
<tr>
<td>Female</td>
<td>56-65</td>
<td>7</td>
<td>5.5</td>
</tr>
<tr>
<td>Female</td>
<td>&gt;65</td>
<td>6</td>
<td>4.6</td>
</tr>
</tbody>
</table>

\[ p = 0.19 \]

*Non-significant trend towards more older subjects than expected.*
## GENDER/AGE DISTRIBUTION

- **Healthy Controls:**

<table>
<thead>
<tr>
<th>Gender/Age Category</th>
<th>Observed</th>
<th>Expected</th>
</tr>
</thead>
<tbody>
<tr>
<td>Male / &lt;56</td>
<td>6</td>
<td>7.1</td>
</tr>
<tr>
<td>Male / 56-65</td>
<td>2</td>
<td>7.4</td>
</tr>
<tr>
<td>Male / &gt;65</td>
<td>7</td>
<td>5.9</td>
</tr>
<tr>
<td>Female / &lt;56</td>
<td>5</td>
<td>3.7</td>
</tr>
<tr>
<td>Female / 56-65</td>
<td>7</td>
<td>3.7</td>
</tr>
<tr>
<td>Female / &gt;65</td>
<td>4</td>
<td>3.1</td>
</tr>
</tbody>
</table>

\[ p = 0.16 \]

*Non-significant trend towards more females than expected.*
## DEMOGRAPHIC CHARACTERISTICS

<table>
<thead>
<tr>
<th></th>
<th>PD Subjects (N = 46)</th>
<th>Healthy Controls (N = 31)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Males</strong></td>
<td>28 (61%)</td>
<td>15 (48%)</td>
</tr>
<tr>
<td><strong>Age (mean)</strong></td>
<td>62</td>
<td>60</td>
</tr>
<tr>
<td>&lt;56 years</td>
<td>10 (22%)</td>
<td>11 (35%)</td>
</tr>
<tr>
<td>56-65 years</td>
<td>20 (43%)</td>
<td>9 (29%)</td>
</tr>
<tr>
<td>&gt;65 years</td>
<td>16 (35%)</td>
<td>11 (35%)</td>
</tr>
<tr>
<td><strong>Hispanic/Latino</strong></td>
<td>0 (0%)</td>
<td>1 (3%)</td>
</tr>
<tr>
<td><strong>Race</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Caucasian</td>
<td>41 (89%)</td>
<td>29 (94%)</td>
</tr>
<tr>
<td>African-American</td>
<td>0 (0%)</td>
<td>1 (3%)</td>
</tr>
<tr>
<td>Other</td>
<td>5 (11%)</td>
<td>1 (3%)</td>
</tr>
</tbody>
</table>
PD SUBJECT CHARACTERISTICS

PD Subjects
(N = 46)

Family Hx of PD  8 (17%)
Mn duration of disease  9 months
Mn MDS-UPDRS score
- Total score 35.7
- Part I 7.0
- Part II 7.3
- Part III (Motor Exam) 21.4
Mn Modified Schwab 92
Hoehn & Yahr
- Stage 1 15 (33%)
- Stage 2 27 (59%)
- Unknown 4 (9%)
PD Subjects:

- **23 protocol deviations (in 22 subjects)**
  - 18 due to eligibility criteria (Most due to DaTSCAN Availability)
  - 1 due to lumbar puncture (CSF testing for hemoglobin)
  - 4 due to DaTSCAN (dosage)

Healthy Controls

- **14 protocol deviations (in 14 subjects)**
  - 8 due to eligibility criteria
  - 1 due to lumbar puncture (CSF testing for hemoglobin)
  - 1 due to MRI not done
  - 4 due to DaTSCAN (dosage)
PD Subjects:

- No early study terminations

Healthy Controls

- 1 early study termination
  - due to ‘other’:
    “Subject unwilling to comply with all study assessments”
    Specifically the lumbar puncture
      - as a result completing the screening LP with CSF collection is required before subject enrolls
REPORTABLE EVENTS

PD Subjects:

- 3 reportable events (in 3 subjects)
  - All due to starting PD meds
    - 6 month visit

Healthy Controls

- 1 reportable event (in 1 subject)
  - Due to early withdrawal
    - subject did not want to complete LP
ADVERSE EVENTS

PD Subjects:

- 7 adverse events (in 3 subjects)
  - 3 LP-related AE’s
    - “Back pain” / “Headache” / “Loss of consciousness”
  - No DaTSCAN-related AE’s

Healthy Controls

- 11 adverse events (in 4 subjects)
  - 6 LP-related AE’s
    - “Abdominal Pain” / “Back Pain” x 2 / “Headache” x 3
  - No DaTSCAN-related AE’s
SERIOUS ADVERSE EVENTS

PD Subjects:
- No serious adverse events observed to date

Healthy Controls
- No serious adverse events observed to date
### PD SUBJECTS EXCLUDED BY DaTSCAN

(N = 6)

<table>
<thead>
<tr>
<th>Family Hx of PD</th>
<th>2 (33%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mn duration of disease</td>
<td>5 months</td>
</tr>
<tr>
<td>Mn MDS-UPDRS score</td>
<td></td>
</tr>
<tr>
<td>• Total score</td>
<td>21.5</td>
</tr>
<tr>
<td>• Part I</td>
<td>6.7</td>
</tr>
<tr>
<td>• Part II</td>
<td>3.0</td>
</tr>
<tr>
<td>• Part III (Motor Exam)</td>
<td>11.8</td>
</tr>
<tr>
<td>Mn Modified Schwab</td>
<td>96</td>
</tr>
<tr>
<td>Hoehn &amp; Yahr</td>
<td></td>
</tr>
<tr>
<td>• Stage 1</td>
<td>3 (50%)</td>
</tr>
<tr>
<td>• Stage 2</td>
<td>3 (50%)</td>
</tr>
<tr>
<td>• Unknown</td>
<td>0 (0%)</td>
</tr>
</tbody>
</table>
## PD SUBJECT CHARACTERISTICS

<table>
<thead>
<tr>
<th>PD Subjects</th>
<th>Enrolled</th>
<th>Excluded</th>
</tr>
</thead>
<tbody>
<tr>
<td>N = 46</td>
<td></td>
<td>(N = 6)</td>
</tr>
</tbody>
</table>

- **Family Hx of PD**
  - Enrolled: 8 (17%)  
  - Excluded: 2 (33%)

- **Mn duration of disease**
  - Enrolled: 9 months  
  - Excluded: 5 months

- **Mn MDS-UPDRS score**
  - Total score: 35.7  
  - Part I: 7.0  
  - Part II: 7.3  
  - Part III (Motor Exam): 21.4

- **Mn Modified Schwab**
  - Enrolled: 92  
  - Excluded: 96

- **Hoehn & Yahr**
  - Stage 1: 15 (33%)  
  - Stage 2: 27 (59%)  
  - Unknown: 4 (9%)  
  - Stage 1: 3 (50%)  
  - Stage 2: 3 (50%)  
  - Unknown: 0 (0%)