PPMI Clinical Data Recap

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PARKINSON'S PROGRESSION MARKERS INITIATIVE
Play a Part in Parkinson's Research

OVERVIEW

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

ENROLLMENT (CTCC REPORT)

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

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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

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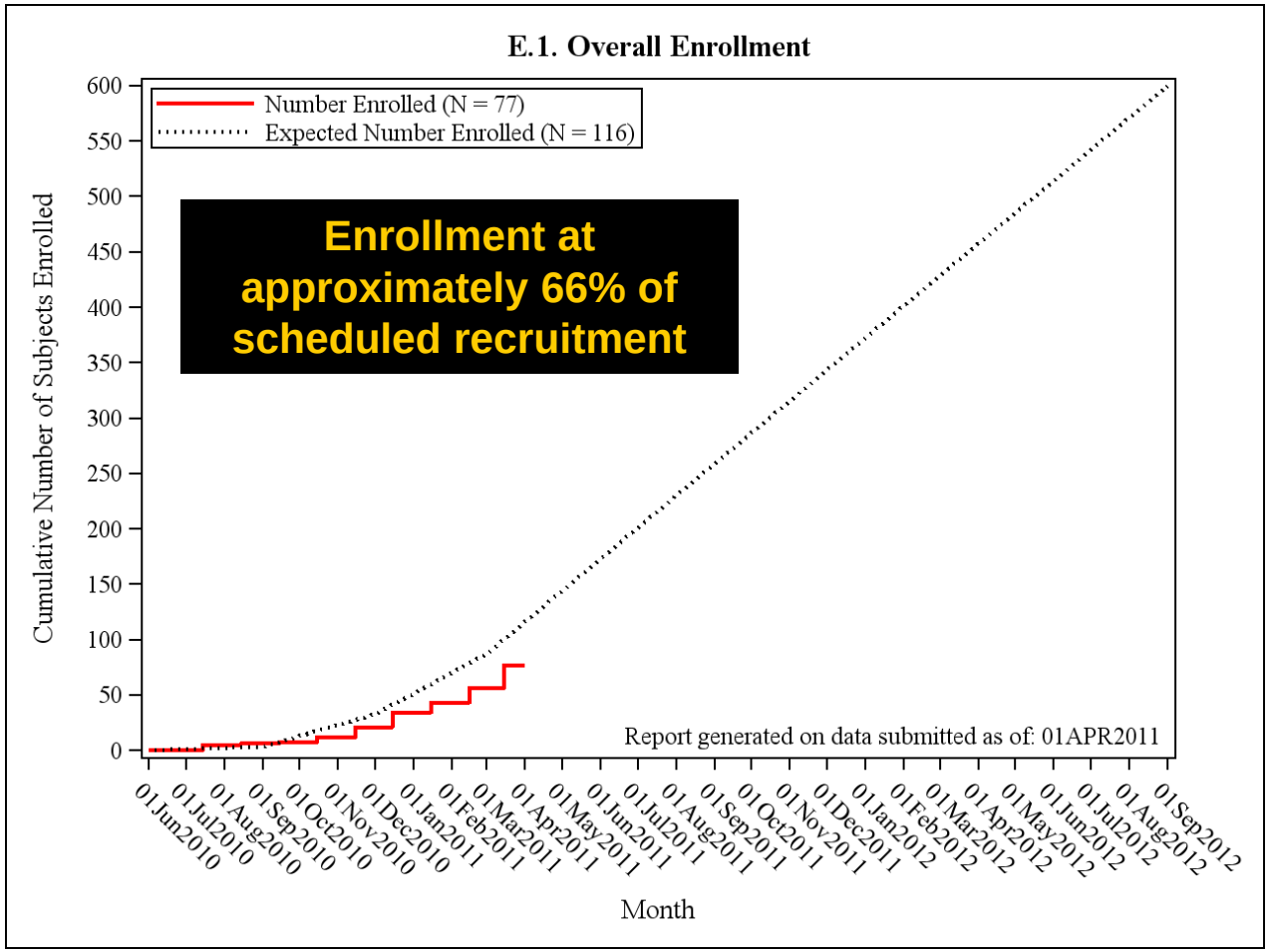
ENROLLMENT (CSOC REPORT)



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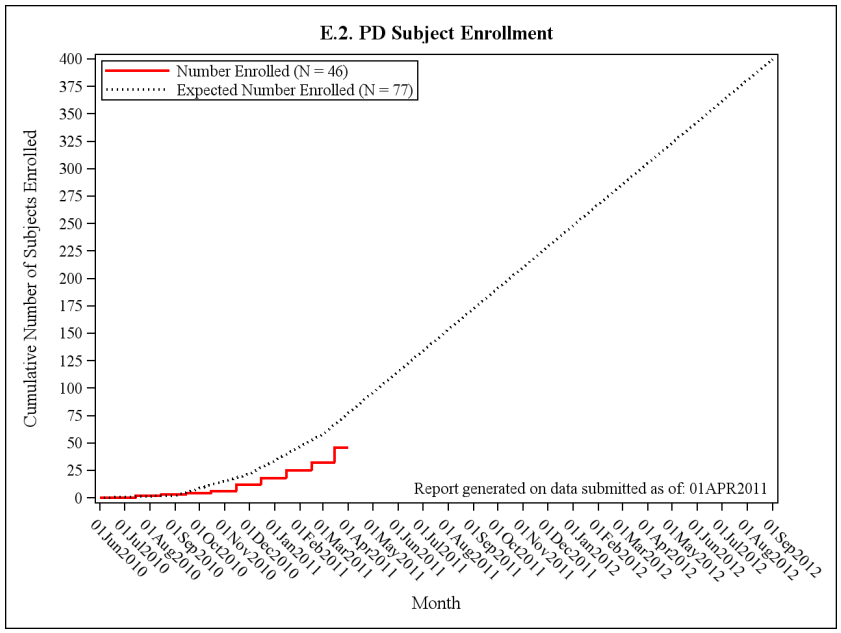

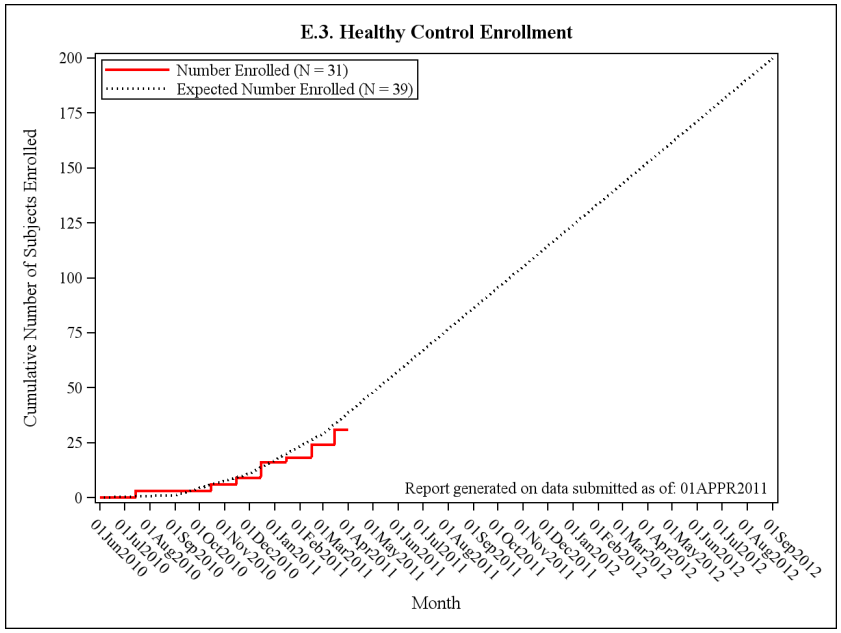

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REASONS FOR EXCLUSION

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)

BASELINE DATA COMPLETENESS

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:

	Observed	Expected
• Male / <56	5	10.6
• Male / 56-65	9	11.0
• Male / >65	14	8.7
• Female / <56	5	5.5
• Female / 56-65	7	5.5
• Female / >65	6	4.6

p = 0.19

Non-significant trend towards more older subjects than expected.

GENDER/AGE DISTRIBUTION

- Healthy Controls:

	Observed	Expected
• Male / <56	6	7.1
• Male / 56-65	2	7.4
• Male / >65	7	5.9
• Female / <56	5	3.7
• Female / 56-65	7	3.7
• Female / >65	4	3.1

p = 0.16

Non-significant trend towards more females than expected.

DEMOGRAPHIC CHARACTERISTICS

	PD Subjects (N = 46)	Healthy Controls (N = 31)
Males	28 (61%)	15 (48%)
Age (mean)	62	60
• <56 years	10 (22%)	11 (35%)
• 56-65 years	20 (43%)	9 (29%)
• >65 years	16 (35%)	11 (35%)
Hispanic/Latino	0 (0%)	1 (3%)
Race		
• Caucasian	41 (89%)	29 (94%)
• African-American	0 (0%)	1 (3%)
• Other	5 (11%)	1 (3%)

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%)

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PROTOCOL DEVIATIONS

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)

EARLY STUDY TERMINATIONS

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)
Family Hx of PD	2 (33%)
Mn duration of disease	5 months
Mn MDS-UPDRS score	
• Total score	21.5
• Part I	6.7
• Part II	3.0
• Part III (Motor Exam)	11.8
Mn Modified Schwab	96
Hoehn & Yahr	
• Stage 1	3 (50%)
• Stage 2	3 (50%)
• Unknown	0 (0%)

PD SUBJECT CHARACTERISTICS

PD Subjects	Enrolled (N = 46)	Excluded (N = 6)
Family Hx of PD	8 (17%)	2 (33%)
Mn duration of disease	9 months	5 months
Mn MDS-UPDRS score		
• Total score	35.7	21.5
• Part I	7.0	6.7
• Part II	7.3	3.0
• Part III (Motor Exam)	21.4	11.8
Mn Modified Schwab	92	96
Hoehn & Yahr		
• Stage 1	15 (33%)	3 (50%)
• Stage 2	27 (59%)	3 (50%)
• Unknown	4 (9%)	0 (0%)