PPMI – GOALS 2019

Ken Marek

PPMI Annual Meeting
May 3, 2018
New York, NY
## PPMI STUDY DETAILS: SYNOPSIS

### STUDY POPULATION
- 423 de novo PD subjects (newly diagnosed and unmedicated)
- 196 age-and gender-matched healthy controls
- 64 SWEDD subjects
- 67 individuals with prodromal PD (hyposmic, RBD)
- 550 LRRK2 or GBA (PD manifest and non-manifesting family members)
- 50 Synuclein (PD manifest and non-manifesting family members)
- Subjects followed for 5 to 13 years

### ASSESSMENTS/CLINICAL DATA COLLECTION
- Motor assessments
- Neuropsychiatric/neurobehavioral testing
- Autonomic, olfaction, sleep
  - DaTSCAN, AV133, Amyloid, DTI/RS MRI
- Sensor Data

### BIOLOGIC COLLECTION
- DNA, RNA
- Serum, whole blood and plasma collected at each visit; urine annually
- CSF collected at baseline, 6mo 12mo and then annually
- IPSC in subset
- Samples aliquotted and stored in central biorepository
- Post mortem tissue

### Shared Data and Biosamples
- > 1,700,000 Data downloads
- > 100 Sample requests via BRC
- Ancillary study development
DATA

CLINICAL

IMAGING

BIOLOGICS

GENETICS

WEARABLES
DNA – METHYLATION
RNA SEQ
OMICS
Meeting Goals - Review study Success/Challenges

- Continued outstanding retention
- Continued compliance clinical, imaging, biofluid data
- Add to robust biofluid biorepository
- Report on longitudinal analysis of progression data
  - PD subsets, prediction of progression
  - Integrate data sets Imaging, Genetics, Wearables
- Develop tools for Prodromal assessments – phenoconversion, pre-motor progression
- Pathology core
- Long-term PPMI follow-up – FOUND
- Wearable/sensor technology
- How can PPMI inform clinical trials
Goals for PPMI – Think BIGGER

- Can we assess a Prodromal/Preventive Cohort
- How to genotype thousands and PD patients and potential at-risk subjects
- How to develop biofluid biomarkers that inform disease progression – AMP
- How to assess imaging tools focused on disease pathology
- How to use sensor technology to better assess study subjects
- How to take advantage of onset of disease milestones – cognition, gait, dyskinesia
- How can PPMI utilize Fox Insight