

Ancillary Studies



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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



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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

The screenshot displays the PPMI Ancillary Studies section of the website. At the top, there is a navigation bar with 'Parkinson's Progression Markers Initiative' on the left and 'Subscriber News' on the right. Below this is a search bar with the text 'Enter search keyword' and a 'search' button. A 'Contact Us Home' link is also present. A secondary navigation bar includes 'Study Design', 'Research Documents & SOPs', 'Stats Forum', and 'Ancillary Studies'. The main content area is titled 'ANCILLARY STUDIES' and contains several paragraphs of text explaining the submission process, including criteria for review and the requirement for a Letter of Intent (LOI). To the right of the text are four icons with labels: 'DOWNLOAD DATA', 'REQUEST SPECIMENS', 'for PROSPECTIVE PARTICIPANTS', and 'for PRACTITIONERS'. Below the text is a section titled 'Ancillary Studies Proposal Form' with a note: 'To begin the first stage of Ancillary Studies submission process, complete the form below. * required field'. The form consists of the following fields: 'Principal Investigator First Name*', 'Principal Investigator Last Name*', 'Suffix', 'Position Title', 'Institution*', 'Department', 'Sector' (with a dropdown menu showing 'Please select one'), 'Street Address', 'City*', 'Country*', and 'State*'. On the far right, there are two more icons: 'for INDUSTRY PARTNERS' and 'for RESEARCHERS'.

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.



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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



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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



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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)



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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



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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



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Baseline Demographics (SWEDDS)

Characteristic (n = 25)	Mean	Range; s/d
Age (years)	58.4	38 – 75; 10.9
Gender (percent male)	52%	N/A
Symptom duration (months)	9.5	1.0 – 37.0; 9.2
MDS-UPDRS, part III score	16.2	3 – 39; 9.7
Tremor (percent present)	84%	N/A
UPSIT (raw score)	30.8	12 – 39; 6.6
Number of PPMI sites with at least one SWEDD	11	N/A



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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.



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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



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TAP-PD baseline demographics

Characteristic (n= 10)	Mean	Range
Age	62	50-81
Gender (% male)	80%	
OMS	41.2	24.1-48.6
Key Down	3.9	3.4-4.3
Peg Cycle	0.9	0.8-1.4



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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



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