Research Into PSP/CBD: 2018 and Beyond

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PSP and CBD

Outline

• Observational studies - overview
• Theoretical considerations
• Importance of biomarkers
• Observational studies - protocols
  • Molecular Anatomic Imaging Analysis
  • ARTFL Protocol
  • 4RTN12 Protocol
  • LEFFTDS Protocol
• Focus on tau
• Clinical trials
• Additional words of wisdom

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Observational Studies - Overview
Theoretical considerations for experimental drug trials

- Slowed progression with disease-modifying therapy
- Halted progression with disease-modifying therapy
There is a critical need to study the natural history of PSP and CBD to prepare for future therapies. Observational studies include:

- Clinical testing
- Neuropsychological testing
- MRI scans
- PET scans
- Blood samples
- CSF samples
- Neuropathologic examination

Boxer et al, Lancet Neurol 2014
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**MRI**

- "hummingbird sign" in PSP

- Josephs et al., Mov Disord 2013

- Zalewski et al., J Neurol 2014

**FDG (glucose) PET**

- Zalewski et al., J Neurol 2014
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Importance of Observational Studies

Uncertainties:
- Variability across persons
- Variability over time within individual persons
- Which measures are best for trials?

Aims:
- Determine the best measures that track disease progression (maximize science, minimize time and cost)
- Determine the easiest measures to do the least number of times (minimize burden, time and cost)

Goal:
Prepare for trials which will identify 1 or more treatments that halt or slow disease progression as quickly, cheaply and easily as possible

If you, or a loved one, is participating in research:

THANK YOU FOR PARTICIPATING!

If you, or a loved one, desires to get involved in research:

THANK YOU FOR CONSIDERING!
A Molecular Anatomic Imaging Analysis of tau in Progressive Supranuclear Palsy

- **Study leaders**: Drs. Keith Josephs and Jennifer Whitwell
- **Study coordinator**: Sarah Boland (507-293-4707)

This study will improve understanding of disease progression in PSP which is critically important for the development and testing of future treatments that will likely target tau.

https://clinicaltrials.gov/show/NCT02605785

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Advancement of Research in Frontotemporal Lobar Degeneration

- **Study leaders**: Drs. Adam Boxer and Howard Rosen
- **Study coordinator**: Ping Wang, MA  415-502-7518  pwang@ucsf.edu

This study will improve understanding of disease progression in PSP and CBD (and other FTLD disorders) which is critically important for the development and testing of future treatments that will likely target tau.

https://clinicaltrials.gov/show/NCT02365922

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4 Repeat Tauopathy Neuroimaging Initiative – 2 (4RTNI2)

- **Study leader**: Dr. Adam Boxer
- **Study coordinators**: Dan N Luong  (415) 476-9578  Phi.Luong@ucsf.edu
  Hilary Heuer, PhD  (415) 476-6743  Hilary.Heuer@ucsf.edu

This study will improve understanding of disease progression in PSP and CBD which is critically important for the development and testing of future treatments that will likely target tau.

https://clinicaltrials.gov/show/NCT02966145
Longitudinal Evaluation of Familial Frontotemporal Dementia Subjects

- **Study leaders**: Drs. Brad Boeve and Howard Rosen
- **Study coordinator**: Leah Forsberg 507-293-5551 forberg.leah@mayo.edu
- This study will improve understanding of biomarker and disease progression in familial FTD in those who have a mutation in MAPT (tau), progranulin or C9orf72.

https://clinicaltrials.gov/show/NCT02372773

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**Focus on Tau**

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**Focus on Tau**
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Clinical Trials

https://www.psp.org/iwanttolearn/research/

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Research

CurePSP: Breakthroughs into the battle against PSP, CBD, and other disorders have now taken a new direction, catalyzed by a number of recent discoveries, exciting emerging work, and new treatments. Please watch this video to learn more about CBD and CBD research. On November 12, 2015, a panel of eminent CurePSP grantees, moderated by Dr. Robert Weiss, prominent author, Jonathan Weiner, discussed the latest development in neurodegeneration research.

https://www.psp.org/ineedsupport/research/

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Clinical Trials

https://www.psp.org/ineedsupport/clinical-trials/
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Clinical Trials

- Contact an MD, study coordinator or site to discuss details of the trial

- Procedures:
  - Note strict criteria for enrollment (age, overall health, medications, severity, etc.)
  - Must be prepared to commit to the entire trial
  - Written informed consent form provides all details
  - Screening visit is key
  - Duration usually 3 months to 2 years
  - Visits vary from every 2 to 12 weeks
  - Clinical testing, questionnaires, blood, EKG, MRI
  - CSF exam and/or PET scans (depending on the study)
  - Strong focus on safety

- Enormously important for scientific advancement

- May impact one’s own symptoms or progression

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Additional Words of Wisdom
(from patients and families)

- Non-medication approaches are as or more important than currently available medications
- Patients and care partners - stay mentally active, socially active, physically active
- Divert frustrations to the illness and not the person who is facing the challenges
- Maintain a sense of humor
- Learn and practice mindfulness
• Strive for optimizing one’s purpose in life despite the illness

• Thank you for your attention
• Thank you for participating, or considering to participate, in research
• Thank you for your involvement/participation with CurePSP
• Maintain the Fight!