Prime of Life
Brain Diseases

Research Gateway to Treatment and Cure of Neurodegeneration
Finding a cure for PSP would transform the lives of thousands while giving hope to the millions who suffer from related brain disorders.

John Q. Trojanowski, MD, PhD
Co-Director, Center for Neurodegenerative Disease Research, University of Pennsylvania

A true story

It started with uncharacteristic behavior—use of juvenile language, wearing showy hats, racing to cross streets ahead of changing lights at dangerous New York City intersections. Mary, a beautiful, spirited and stylish woman in her mid-fifties, was a prominent Wall Street executive, the wife of a successful private-equity fund manager, the mother of two college-aged sons, an active sportswoman, a curious traveler, a leader in charitable and professional organizations and a gardener known for her green thumb tending to the family’s country home in upstate New York.

As the symptoms progressed, her husband grew concerned. Mary exhibited inappropriate behavior in social situations; there were conduct issues at her investment firm, where she was a top executive. Her marriage had always been loving and caring, but her husband and children increasingly came under intense pressure as she became irrationally critical and upset.
With the condition worsening, Mary had difficulty sleeping, hallucinations, mobility problems with stiffness, falls that cracked ribs. Cognitive issues progressed—during an overnight hotel stay, her husband was alerted by the desk clerk that she was wandering outside barefoot and in her nightgown.

Desperately, Mary’s husband sought treatment. As dramatic as her symptoms were, more than a dozen neurologists either gave no diagnosis or the wrong one. Drug treatments seemed to make matters worse. The family spent tens of thousands of dollars in naturopathic therapies to no avail. The condition continued its inexorable march toward mortality.

Toward the latter stages of the disease, Mary was immobile, unable to speak and required around-the-clock nursing care, yet evidently had comprehension of what was being said and what was going on around her. She was a prisoner in her own body. Her inevitable death came peacefully at age 61. An autopsy determined the condition to be corticobasal degeneration (CBD), a rare and invariably fatal neurodegenerative condition that is one of several diseases in the prime of life spectrum.
Neurodegeneration that strikes when we least expect it

Prime of life neurodegeneration is a spectrum of relatively rare diseases that often afflict people during productive, active years and lead to debilitating symptoms and early death. They are generally categorized as frontotemporal disorders and are characterized by progressive personality, behavior, language and motor decline. These diseases create a level of functional impairment that significantly compromises the ability to carry out activities of daily living. There is currently no treatment and no cure, but there is hope. Research into these diseases may be a gateway to understanding and ultimately preventing, treating and curing more common neurodegenerative diseases like Alzheimer’s and Parkinson’s.

CurePSP is the leading organization within this disease spectrum, providing support for patients, families and caregivers; awareness and education to healthcare professionals; and global research funding. Current research into prime of life diseases, especially progressive supranuclear palsy (PSP), is showing great promise for unlocking the secrets of neurodegeneration.

Prime of life brain diseases:
- Progressive Supranuclear Palsy (PSP)
- Corticobasal Degeneration (CBD)
- Multiple System Atrophy (MSA)
- Frontotemporal Dementia (FTD)
- Amyotrophic Lateral Sclerosis (ALS)
- Chronic Traumatic Encephalopathy (CTE)

The attention of top researchers, biotech firms and drug companies is rapidly turning to PSP. This is a disease where new basic science ideas are translating into drug trials as never before.

Lawrence I. Golbe, MD
Director, Division of Movement Disorders
Program Director, Neurology Residency
Rutgers Robert Wood Johnson Medical School

Neurodegeneration that strikes when we least expect it
The key to unlocking the secrets of brain disease

CurePSP is a leader in supporting research into PSP and CBD. Findings from these studies and others suggest that mechanisms in PSP, CBD and other prime of life diseases are also involved in Alzheimer’s and Parkinson’s diseases. Common factors include gene mutations, protein pathology and environmental influences. Research funded by CurePSP and others continues to investigate genetics, stem cell and drug therapies, disease-causing proteins (prions), the role of abnormal tau aggregation and symptomatic manifestations and biomarkers.
The central role of PSP in neurodegeneration research

Research into PSP is an ideal gateway to understanding the causes of other neurodegenerative diseases. It delivers potentially wide-ranging benefits in proportion to money invested. There are several reasons:

1. Reliable diagnosis as compared to other neurodegenerative diseases provides an identified patient population for clinical trials.

2. Smaller patient populations require smaller sample sizes for statistically significant results.


4. Many neurodegenerative diseases are characterized by tau abnormalities (tauopathies) but only a few, like PSP, involve only the tau protein. This allows researchers to target tau.

5. CurePSP’s support of the Eloise H. Troxel Memorial Brain Bank at the Mayo Clinic helps to fund distribution of postmortem brain samples to researchers.

6. The global PSP Genetics Consortium, a joint venture of CurePSP and the Tau Consortium, is identifying genetic mutations associated with PSP.

7. CurePSP’s Patient Engagement Program (PEP) directs patients into clinical trials and helps to reduce dropout rates.

*PSP has proven to be the most attractive target for clinical trials. Treatment for one of these tauopathies is likely to trigger treatment for them all.*

Bruce L. Miller, MD
Director, Memory and Aging Center
University of California, San Francisco
Chair, the Tau Consortium
The research roadmap

CurePSP’s targeted approach to research promises to unlock new discoveries about PSP and related prime of life diseases. Our research roadmap prescribes experimental tools using cellular and animal lab models with markers developed for diagnosis and measurement. This includes identification of drug targets, screening them in molecular and cellular models, testing drugs in vertebrates using preclinical models and clinical trials by our partners in industry and academia.

Identifying genetic risk factors for PSP is like using hand- and footholds while climbing a mountain. The PSP Genetics Consortium will provide pivotal results for finding new drug targets.

Jeffrey S. Friedman, MD, PhD
Managing Director,
PSP Genetics Consortium

Identify most promising drug targets

Procedures

Genetics

Prions

Proteins

Models

Markers

Identify the cellular pathways controlled by the most salient genes from CurePSP’s earlier studies

Identify steps in tau misfolding, templating and cell-to-cell transmission

Identify defects in production, handling and disposal of tau and other proteins in PSP/CBD

Create new cellular and animal lab models more faithful to human PSP/CBD

Devise better tests for diagnosis and measurement of PSP/CBD and its treatment response in humans

Perform high-throughput screens

Test drugs in vertebrate preclinical models

Early-phase clinical trials by industry or academia

Late-phase clinical trials by industry

New Experimental Tools

Scientific Hypothesis

CurePSP’s Patient Engagement Program
Your donation will make a difference

CurePSP is strategically positioned at the forefront of scientific inquiry that promises prevention, treatment and cure of neurodegenerative diseases. We have funded more than 170 research grants since 1997, a commitment that continues today.

CurePSP has donor-named research opportunities that offer involvement in exciting and groundbreaking scientific inquiry in genetics, proteins, stem-cell therapies, animal lab models, drug discovery and early-phase clinical trials. We can discuss your areas of interest and level of involvement desired.

Contributions to our Annual Fund provide discretionary support for research, programs and education. Tribute Gifts are a meaningful way to honor a loved one and planned giving benefactors are recognized through our Legacy Society. Our staff provide comprehensive support for fundraising events.

Please help CurePSP in its quest to find treatment and cure for neurodegeneration. The need is urgent. The time is now.

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