



PRESS RELEASE

Progressive Supranuclear Palsy Phase 2 Platform Clinical Trial announces the selection of AADvac1 and AZP2006 for its first two regimens

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The Progressive Supranuclear Palsy (PSP) Trial Platform (PTP), led by Drs. Adam Boxer (University of California, San Francisco [UCSF]), Irene Litvan (University of California, San Diego), Julio Rojas (UCSF) and Anne-Marie Wills (Massachusetts General Hospital), has selected two promising drug candidates—Axon Neuroscience's AADvac1 and Alzprotect's AZP2006—for inclusion in the platform trial as the first two compounds to be evaluated. The trial aims to accelerate the development of effective treatments for PSP, a rare and fatal neurodegenerative disease. Additional compounds to be evaluated in the trial are expected to be announced later this year.

Funded by the National Institute on Aging (NIA), part of the U.S. National Institutes of Health (NIH), the platform trial is supported by a five-year grant. Its design enables multiple therapies to be tested concurrently, establishing a perpetual, efficient and flexible path to evaluate promising drug candidates.

*"This public-private partnership represents an unprecedented opportunity to accelerate the development of treatments for PSP," said **Professor Adam Boxer**, endowed professor in memory and aging in the UCSF Department of Neurology, as well as a principal investigator for the trial. "By bringing together innovative therapeutic approaches like AADvac1 and AZP2006 in an efficient clinical trial design, we aim to address the urgent needs of patients and their families in less time, at a lower cost and with fewer patients on placebo than traditional clinical trials."*

Scientific Innovations: AADvac1 and AZP2006

AADvac1, developed by **Axon Neuroscience** (Bratislava, Slovakia), is an active immunotherapy targeting pathological tau proteins, which induce and drive PSP and Alzheimer's disease (AD) pathology. The active immunotherapy elicits production of antibodies that bind to abnormal pathological tau, preventing its aggregation and spread, and facilitating its clearance by microglia. In a completed 24-month Phase 2 study in Alzheimer's Disease, AADvac1 demonstrated a favorable safety profile, with therapeutic effects on plasma and cerebrospinal fluid biomarkers and supportive clinical signals showing the potential slowing of disease progression.

*"We are proud and grateful that AADvac1 has been selected by the expert committee for this innovative platform trial," said **Michal Fresser**, CEO of Axon Neuroscience. "We believe the tau-targeting active immunotherapy approach of AADvac1 holds strong potential in the treatment of human tauopathies, and we look forward to partnering with the PSP Trial Platform and the PSP research community to bring forward new treatment options for patients facing a significant unmet medical need."*



AZP2006, developed by **Alzprotect** (Lille, France), is a synthetic small molecule designed to restore lysosomal homeostasis and modulate progranulin (PGRN), while also reducing tau aggregation and neuroinflammation. Preclinical studies demonstrated its ability to correct lysosomal dysfunction in neurodegeneration models. In a 3-month Phase 2a study in PSP patients, AZP2006 demonstrated encouraging clinical and biomarker signals of efficacy. These positive outcomes were further supported by results from a 6-month open-label extension study, reinforcing its therapeutic potential. The compound has received Orphan Drug Designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

“We are honored that AZP2006 has been selected for inclusion in this landmark PSP platform trial,” said **Philippe Verwaerde**, CEO of Alzprotect. *“We are excited to collaborate with the PSP Trial Platform and the broader PSP community to advance innovative therapies for patients with our scientific approach targeting lysosomal dysfunction.”*

About PSP Trial Platform – Collaborative Effort

The trial's steering committee includes researchers: Dr. Adam Boxer (UCSF), Dr. Julio Rojas-Martinez (UCSF), Dr. Anne-Marie Wills (Massachusetts General Hospital), and Dr. Irene Litvan (University of California, San Diego). **CurePSP**—the leading nonprofit advancing research, patient services, and advocacy for PSP and other underserved neurodegenerative diseases—is collaborating with UCSF and approximately 50 trial sites to recruit participants and ensure patient perspectives are integrated throughout the trial. *“I am thrilled to see AADvac1 and AZP2006 enter this landmark platform trial—a powerful, multi-stakeholder collaboration that unites academia, industry, and our community, which we are proud to support,”* said **Kristophe Diaz, PhD**, Executive Director and Chief Science Officer of CurePSP. *“This collaborative platform design can compress years of drug development into real, near-term hope for people living with PSP.”*

Enrollment for the trial is expected to begin at the end of 2025, focusing on patients with Richardson’s syndrome, the most common form of PSP. The trial aims to enroll a population that is fully representative of the US population by providing language support and covering transportation and accommodation costs.

This platform trial represents a collaborative effort among academia, industry, and patient advocacy groups to accelerate the discovery of effective treatments for PSP and improve the lives of those affected by this devastating disease. To learn more about the trial, visit <https://www.psp.org/ptp>.

About Tau Global Conference

Tau Global Conference 2025 brings together three major Tau-focused conferences (Global Tau, EuroTau and CurePSP Neuro), and is hosted by the Alzheimer’s Association, CurePSP and the Rainwater Charitable Foundation. This conference plays a critical role in bringing together interdisciplinary researchers and perspectives to move tau research forward.



About Axon Neuroscience

Axon Neuroscience was founded in 1999 by immunologist Professor Michal Novak. In 1988 Professor Novak discovered tau protein as the major component of neurofibrillary pathology in Alzheimer's disease while working in Laboratory of Molecular Biology, MRC in Cambridge, UK. Axon has been developing active immunotherapy, monoclonal antibody and small molecules for treatment of neurodegenerative diseases and tauopathies.

About Alzprotect

Founded in 2007, Alzprotect is a French Lille-based company committed to the development of innovative therapeutic solutions in the field of neurodegenerative diseases. Including tauopathies, amyloidopathies and synucleinopathies Alzprotect is advancing in the development of AZP2006 (EZEPROGIND), an innovative synthetic molecule administered orally, designed to optimize lysosome homeostasis by regulating both Progranulin and its chaperone protein, Prosaposin.

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