



The PSP Association's International Medical Workshop 7th July 2009

ABSTRACT

Title of Talk: PSP RESEARCH – WHERE NEXT?

Part 1: Speaker(s) details	
Title	Professor
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Part 2: Abstract (Maximum 400 words) Please make your abstract easy to understand as it will appear on our website and will be read by people with PSP and their carers who are not scientists but who will want to understand your work and what it means for them.

Research in PSP showing real results. For some years, our diagnostic methods have been sufficiently accurate for recruiting subjects for clinical trials of drugs that aim to treat only symptoms. However, for trials of neuroprotective drugs (those that slow or halt the underlying disease process), we would prefer study subjects with PSP at its earliest stages, before symptoms develop. This requires much greater sensitivity than present standard diagnostic methods afford, but we are making good progress in developing imaging procedures and biochemical tests of the spinal fluid that would accomplish this. The question then becomes whom to test. Candidates could be relatives of PSP patients or those with falls or with parkinsonism unresponsive to levodopa. Such agents are being developed for Alzheimer's disease, and PSP could benefit via its similarity at with AD at some levels. The Genome-Wide Association Study, a collaboration of scientists in the US, Germany and the UK and supported by CurePSP+, the North American counterpart of PSPA, will be completed by October 2009. It could provide presymptomatic diagnosis and point toward presently unsuspected cellular and chemical pathways contributing to PSP. This in turn could provide new chemical targets for treatment. That project is part of over \$5.2 million in active research grants being supported by CurePSP+. That portfolio illustrates the present direction of PSP research. It includes studies of spinal fluid proteins and electrical responses of the brain as new diagnostic markers; understanding the abnormal function of the H1 tau haplotype (the one genetic variant known so far to be associated with PSP); the effect of microRNA, a new approach to many types of disorders including cancer, on abnormal tau production; the dynamics of abnormal folding and structure of the tau protein; and several treatment trials. The latter emphasize inhibition of abnormal aggregation of tau and include treatment of animal models with methylene blue, grape seed extract, and a new inhibitor of tau phosphorylation called TTT-3002. There are also trials in humans of Coenzyme Q-10, a novel tau aggregation inhibitor called AL-108, noninvasive magnetic stimulation of the brain, and deep brain stimulation in the pedunculopontine nucleus, an area of the brainstem responsible for balance. All of these projects are due for completion by 2011.