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TauRx starts global trial of drug for dementia

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TauRx initiates clinical trial of drug for Frontotemporal dementia

Singapore: TauRx Therapeutics, which is headquartered in Singapore, has initiated a global phase III clinical trial in a type of Frontotemporal Dementia (FTD) also known as Pick's Disease. This form of dementia is similar to Alzheimer's Disease, except that it tends to damage different areas of the brain and affects people as early as 40 years old.

The study focuses on a type of FTD known as behavioral-variant, or bvFTD, which can cause early changes in personality and loss of empathy. A large percentage of these patients have a specific pathology that involves abnormal collections of tau protein in the brain.

The study drug, LMTX, targets a process in the brain whereby a normal form of tau protein begins to self-aggregate due to binding neuronal waste-products. Once the process has started, the aggregates are able to propagate themselves indefinitely, using up normal tau protein and converting it into the toxic aggregates. After destroying the nerve cells where they are initially formed, the aggregates go on to infect nearby healthy neurons, progressively spreading and accelerating the destruction throughout the brain. LMTX stops this aggregation process in its tracks and releases the trapped tau protein in a form which can be easily cleared by nerve cells.

If successful, this will be the first investigational drug that is able to arrest the progression of this disease. TauRx Therapeutics, spun out of the University of Aberdeen, developed the novel treatment based on an entirely new approach which targets aggregates of abnormal fibres of tau protein that form inside nerve cells in the brain.

In a pilot series of cases, LMTX was found to arrest the progression of the disease. LMTX has been found to act in a similar way on the aggregation of TDP-43 protein. Tau or TDP-43 aggregates each account for about 50 percent of patients with this early form of dementia.

Speaking to patients and caregivers at the FTD conference, held between September 5 and 7, in Manchester, UK, Professor

Bradley Boeve of the Mayo Clinic in the US, one of the investigators of the study, said: "Clinicians devoted to FTD clinical trial development have been refining the measures to use in an experimental trial in FTD spectrum disorders for years, and frankly have been waiting for a promising agent. The basic science data for this agent, particularly in the tauopathies, looks sound and the excitement among investigators and among families is high."

The phase III double-blind placebo-controlled study is designed to evaluate the safety and efficacy of LMTX, the second-generation Tau Aggregation Inhibitor (TAI) developed by TauRx. The study aims to confirm the results first seen in the pilot cases in a larger controlled clinical trial in bvFTD patients over a 52-week time-frame. Participating study sites are located in Canada, US, UK, Germany, The Netherlands, Australia and Singapore. Because the condition is relatively rare, TauRx was granted orphan designation for LMTX in 2010, which provides a basis for more rapid approval for marketing if the trial is successful.

"This is an important step forward in our quest to find an effective treatment, with a goal to actually arrest the progression of the disease," said Professor Claude Wischik, founder and CEO of TauRx Therapeutics and Professor of Old Age Psychiatry at the University of Aberdeen. "We are building on over thirty years of research, and the encouraging results from our previous phase II clinical trial in Alzheimer's Disease, which is also correlated with abnormal tau aggregates in the brain."

TauRx previously tested rember, the first-generation TAI on which LMTX is based, in a phase II clinical trial involving 321 patients with mild and moderate Alzheimer's Disease in the UK and Singapore. This study found a 90 percent reduction in the rate of disease progression over two years in Alzheimer's Disease. Professor Wischik and his team have spent nearly 24 years investigating the structure and role of Tau tangles in the development of Alzheimer's disease, FTD and other neurodegenerative diseases. They were the original discoverers of the Tau protein pathology of Alzheimer's.

"It's very exciting news that a treatment is being tested for FTD in a clinical trial," said Penelope Roques of the Frontotemporal Dementia Support Group in the UK. "This is encouraging progress in a disease where there is currently no treatment available." The group has about 1,000 members across the UK, ranging from FTD patients, caregivers and family members.