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TauRx's LMTXs gets FDA Orphan-drug Designation

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LMTXÃ?Â® is the first protein aggregation inhibitor to reach Phase 3 clinical development for treatment of neurodegenerative diseases of the brain and was developed by TauRx based on nearly 30 years of research.



TauRx, a leader in neurodegenerative disease research, announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to LMTX® for the "treatment of frontotemporal dementia".

The term Frontotemporal Dementia (FTD) covers a number of sub-types of dementia, all of which are characterized by a progressive loss of neurons in the frontal and/or temporal lobes of the brain.

LMTX® is the first protein aggregation inhibitor to reach Phase 3 clinical development for the treatment of neurodegenerative diseases of the brain and was developed by TauRx based on nearly 30 years of research. Protein aggregation inhibitors work by undoing the protein aggregations in the brain that are now widely accepted to drive the development of dementia.

This unique mechanism of action can potentially slow the rate of spread of the underlying disease pathology and thereby help to preserve patients' cognition, function and quality of life. Primarily focused at tangled sub-units of the tau protein, the active moiety in LMTX® has also shown to be active against aggregations of the TDP-43 protein; tau and TDP-43 aggregation is causative in most of the FTD sub-types.

"The FDA orphan drug designation of LMTX® is another positive step for patients, clinical researchers and care teams, and it reinforces our continuing research for a disease-modifying treatment for this poorly-addressed family of rare neurodegenerative conditions," said Prof. Claude Wischik, Executive Chairman of TauRx and Professor of Old Age Psychiatry at the University of Aberdeen. LMTX® was granted Orphan Designations in this field by the EMA in 2010.

The FDA only grants ODD status to medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. ODD provides an incentive for sponsors to develop products for rare diseases.