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Development of FAF1 inhibitor KM-819 as a disease-modifying drug for treatment of Parkinson's disease

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Current standard of care for Parkinson's disease is symptomatic treatments by supplementing dopamine or dopamine agonists or analogous mechanisms, and the disease modifying treatment is one of the major unmedical needs to block the progression. KM-819 is an orally active small molecule drug developed as an inhibitor for FAF1, a proapoptotic protein, targeting various degenerative diseases. It has shown superior efficacy of neuroprotection in cell models and of dopaminergic neuron protection in midbrain in various animal models of Parkinson's disease as well as improvement of behavioral tests, suggesting this drug has potential capability of slowing or stopping the progression of the disease. It has also shown inhibition of alpha-synuclein accumulation in cells. We have completed Phase 1 clinical trial for KM-819, randomized, double-blind, placebo-controlled study. The study is divided into part A (single ascending dose) and part B (multiple ascending dose) for evaluation of safety, tolerability, and pharmacokinetics as well as various pharmacodynamics markers for KM-819 in healthy volunteers. The study results showed no drug-related serious AEs and high safety profile in human. Also, the PK study showed dose-proportional exposure with higher in elderly group, ideal for Parkinson's drug. We are currently planning for phase 2 in patients focusing on investigation of the drug's efficacy of slowing down or halting the progression of the disease.

Biography

Jae Moon Lee completed his PhD from Duke University and Post-doctorate from Duke University School of Medicine. He is the VP of Kainos Medicine, a clinical stage Korean biotech company. He has published more than 15 papers in reputed journals.

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