Journal of Clinical Medicine Publishes Results of Phase 2 Trial of Nilotinib in Treatment of Dementia with Lewy Bodies

Dementia with lewy bodies ("DLB") patients, treated with 200mg of nilotinib had statistically significant improvement in cognitive function compared to placebo.

MCLEAN, VA, UNITED STATES, June 25, 2025 /EINPresswire.com/ -- Nilotinib demonstrated a statistically significant improvement in cognitive function compared to placebo as measured by ADAS-COG14 with a compelling safety profile

KeifeRx announced this week that the full results of a phase 2 trial of nilotinib for the treatment of patients with Lewy Body Dementia conducted in collaboration with Georgetown University Medical Center and MedStar Health have been published in the Journal of Clinical Medicine.

The results showed that in patients with dementia with Lewy bodies ("DLB"), treatment with 200mg of nilotinib resulted in a statistically significant improvement in cognitive function and a 73% reduction in falls compared to placebo.

About the Trial:

The phase 2 randomized, double-blind study (NCT04002674) evaluated the safety and tolerability of nilotinib in individuals with DLB. The trial included 43 adults who were randomized to either receive nilotinib (200 mg/day) or placebo over a 6-month period. The clinical endpoint was the change in ADAS-Cog14 total score. Patients receiving nilotinib showed a greater improvement in cognitive decline as measured by ADAS-Cog14 with a 2.8-point improvement over 3 months (95% CI, p=0.037) and 3.2 points over 6 months (95% CI, p=0.08)

Safety measures showed more adverse events in the placebo group (74) versus nilotinib (37) groups (p=.0054) and the number of falls was reduced in the nilotinib group (6) versus placebo (21) group (p=.006) by 73%.

The study documented an improvement in homovanillic acid (HVA), a biomarker of dopamine levels, (p=.004) for those taking nilotinib and a reduction in the ratio of pTau181/AB42 (p=.0034), which the authors say suggest a reduced CNS amyloid burden. There was also a positive effect on CSF markers of inflammation.

The study showed that Nilotinib demonstrated favorable safety, biomarkers and efficacy outcomes in patients with dementia with Lewy bodies. The study authors further noted that the favorable effects of nilotinib (200 mg) on cognition, without worsening motor and/or behavioral symptoms, may help overcome the dilemma of currently approved symptomatic drugs that improve motor but worsen cognitive symptoms, or vice versa.

“These ground-breaking clinical trial results demonstrate the potential of nilotinib to provide the first effective treatment addressing dementia in LBD patients without negatively impacting movement or other symptoms,” said KeifeRx CEO Chris Hoyt. “We are encouraged by the results and believe that nilotinib may be an effective treatment for the hundreds of thousands of DLB patients suffering from dementia in the United States.”

KeifeRx, a biopharmaceutical company located in McLean, Va. holds an exclusive license from Georgetown University for a patent associated with the use of nilotinib in neurodegenerative diseases, including DLB, Alzheimer’s disease, and Parkinson’s disease dementia. The study’s senior author, Charbel Moussa, PhD, MBBS, is a named inventor on the patent.

“As a scientist, the opportunity to use an existing, well-studied drug like nilotinib as a baseline for development of the next generation of therapies for neurodegenerative dementias is a dream. This study provided further evidence that nilotinib can make a meaningful contribution to slowing the progression of dementia and the associated complications like falls,” said Moussa, associate professor of neurology at Georgetown University Medical Center and Director of the Translational Neurotherapeutics Program.

“The findings from this Phase II trial suggest that Nilotinib could become a vital treatment for patients with Dementia with Lewy Bodies and other forms of dementia including Parkinson’s Disease Dementia,” said the study’s first author Fernando Pagan, MD, associate professor of neurology at Georgetown University Medical Center, vice chairman of neurology at MedStar Georgetown University Hospital and medical director of the Translational Neurotherapeutics Program. “As a neurologist specializing in neurodegenerative disease, I look forward to offering hope to patients where current therapies fall short. I am optimistic that nilotinib could have a positive impact on the standard of care for these patients.”

About KeifeRx

KeifeRx is an emerging clinical-stage biopharmaceutical company developing a portfolio of novel and optimized, low-dose kinase inhibitors for the treatment of multiple, high-need neurodegenerative and immune diseases, including Alzheimer’s disease, Parkinson’s disease, amyotrophic lateral sclerosis (ALS), and dementia with Lewy bodies. KeifeRx’s diverse pipeline of early- and late-stage products leverages mechanisms of action inherent to kinase inhibitors which thus far have been underexplored. This includes the ability to penetrate the brain, induce autophagy, and enable the bulk disposal of disease-causing toxic proteins to treat

neurodegenerative diseases. Georgetown University owns several issued patents and pending patent applications on the underlying technology related to the use of kinase inhibitors for the treatment of neurodegenerative diseases with KeifeRx co-founder Dr. Charbel Moussa, MBBS, Ph.D., named as one of three inventors. Dr. Charbel Moussa is a co-founder, shareholder and paid consultant to KeifeRx. Dr. Fernando Pagan is a co-founder, shareholder and board member of KeifeRx. KeifeRx has an exclusive license to intellectual property from Georgetown University. For more information on KeifeRx, please visit https://www.keiferx.com.

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