Umecrine Cognition receives a grant from The Michael J. Fox Foundation to support preclinical evaluation of golexanolone in Parkinson's disease

STOCKHOLM – May 5, 2025. Umecrine Cognition today announces that the company has been awarded a research grant by The Michael J. Fox Foundation for Parkinson's Research (MJFF). The funding will be used to conduct preclinical studies to evaluate the potential treatment effect of golexanolone in Parkinson's disease. The grant was awarded to the collaboration between Umecrine Cognition and the Principal Investigator Professor Gilberto Fisone at Karolinska Institutet, Solna, Sweden, and will be disbursed in three installments over the coming two years.

Parkinson's disease (PD) is a progressive neurodegenerative disease most noticeably characterized by deteriorating motor functions. However, non-motor symptoms, such as sleep disorders and cognitive impairments, emerge before the onset of physical symptoms and have, historically, been overlooked due to a lack of scientific and clinical insights. While current treatments target motor dysfunction, there are no approved pharmaceutical therapies for non-motor symptoms. However, breakthrough results from previous preclinical studies show that golexanolone has a positive impact on non-motor symptoms. The USD 420,000 grant will support our preclinical studies to confirm golexanolone's treatment effect on Parkinson's-related sleep dysfunction and cognitive impairments. Furthermore, the grant will support preclinical studies to evaluate the drug candidate's effect on disease progression in several disease models.

The project aims to confirm the clinical feasibility of golexanolone, a novel drug candidate in Phase 2 clinical development, as a treatment for non-motor symptoms in PD. This will be done using a novel disease model of PD [1], developed by Professor Gilberto Fisone, Head of the Laboratory of Molecular and Circuit Neuropharmacology, and Chair of the Department of Neuroscience, at Karolinska Institutet. This model reproduces excessive daytime sleepiness and concomitant alterations in sleep micro-structure – as observed in patients – and cognitive dysfunction. Further, following results indicating that golexanolone reduces neuroinflammation [2], the project will evaluate golexanolone's effect on symptoms and disease progression in models of alpha-synucleinopathy.

"We are very thankful to MJFF for funding our research efforts. The grant, awarded through MJFF's Parkinson's Disease Therapeutics Pipeline Program, supports the continued evaluation of golexanolone as a potential therapeutic approach to address unmet needs in the clinical management of Parkinson's disease," said Dr. Magnus Doverskog, Umecrine Cognition's Chief Scientific Officer and Co-Principal Investigator.

"Up to 80 percent of patients with Parkinson's disease suffer from sleep disturbances, leading to social dysfunction and reduced quality of life. Despite their high prevalence, these conditions are still poorly understood and there are yet no satisfactory drug treatments. We are therefore very excited to be able to investigate golexanolone, and grateful to MJFF for giving us a unique opportunity to realize this highly translational project," says Professor Gilberto Fisone, Principal Investigator and Chair of the Department of Neuroscience at Karolinska Institutet.

"The Michael J. Fox Foundation supports a broad portfolio of research aimed at exploring novel therapeutic approaches for Parkinson's disease," said Jessica Tome Garcia, PhD, lead scientific program manager of translational research, MJFF. "As part of these efforts, Umecrine Cognition's study focuses on addressing non-motor symptoms in PD, targeting sleep disturbances and disease progression, giving us another avenue to push for meaningful improvements to quality of life for people living with Parkinsons."

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About Parkinson's Disease
Nearly one million Americans currently live with Parkinson's (PD) and approximately 90,000 more receive diagnosis of the disease every year. In Europe, between 900,000-1,900,000 people are estimated to have Parkinson's with another 80,000 to 140,000 new cases every year.

PD is a progressive neurodegenerative disorder primarily characterized by the loss of neurons that produce the neurotransmitter dopamine (dopaminergic neurons) and release it in a brain region that helps control movement (striatum). The loss of dopamine in this area of the brain leads to subsequent motor dysfunction. Clinically, PD presents with hallmark motor symptoms, including resting tremor, slow movement (bradykinesia), rigidity, and postural instability. However, non-motor symptoms – such as sleep disorders, cognitive impairment, autonomic dysfunction, and mood disturbances – are increasingly recognized as significant contributors to the disease burden.

Molecularly, the precise etiology of PD remains incompletely understood, but both genetic and environmental factors are deemed important to the disease progression. Mutations in genes such as GBA1, LRRK2, and SNCA have been implicated in inherited and sporadic forms of PD – with GBA1 mutations specifically linked to an increased risk of cognitive decline. Pathologically, PD is defined by the accumulation of the protein alpha-synuclein, which over time produces neurotoxic Lewy bodies and widespread neuroinflammation. Despite advances in symptomatic treatments, disease-modifying therapies remain an unmet clinical need, underscoring the urgency for novel therapeutic strategies.

About Umecrine Cognition
Umecrine Cognition AB is developing a completely new class of drugs for the treatment of symptoms in the central nervous system related to chronic neuroinflammation – a devastating brain distortion that can lead to severely impaired cognition and fatigue. Chronic neuroinflammation can occur as a result of a number of underlying conditions, including a range of liver diseases as well as neurodegenerative diseases, such as Parkinson's disease. Results from an

internationally acclaimed Phase 2 clinical study indicate that the company's most advanced drug candidate, the GABAA receptor-modulating steroid antagonist golexanolone, normalizes brain signaling and improves cognition and alertness in patients with hepatic encephalopathy. A Phase 2 study is currently ongoing in patients with primary biliary cholangitis. Further, based on intriguing preclinical data, the company is considering pursuing the development of golexanolone in patients with Parkinson's disease. For more information, visit www.umecrinecognition.com.

References:

[1] Medeiros DC, et al., 2023. A mouse model of sleep disorders in Parkinson's disease showing distinct effects of dopamine D2-like receptor activation. Prog Neurobiol. 231:102536. doi: 10.1016/j.pneurobio.2023.102536.

[2] Izquierdo-Altarejos P, et.al., 2024. Golexanolone reduces glial activation in the striatum and improves non-motor and some motor alterations in a rat model of Parkinson's disease. Front Aging Neurosci. 16:1417938. doi: 10.3389/fnagi.2024.1417938.

Attachments

Umecrine Cognition receives a grant from The Michael J. Fox Foundation to support preclinical evaluation of golexanolone in Parkinson's disease