Umecrine Cognition completes interim sample size analysis in Phase 1b/2a study of golexanolone

STOCKHOLM – June 23, 2026. Umecrine Cognition today announced that it has completed an interim sample size analysis in its phase 1b/2a clinical study of golexanolone. The analysis concludes that patient recruitment can continue as planned with no increase in the pre-defined study size. Results are expected in the 2nd part of 2026.

Umecrine Cognition is currently running a randomized, double-blind, placebo-controlled phase 1b /2a study evaluating golexanolone in patients with primary biliary cholangitis, PBC, who experience clinically significant and debilitating fatigue and cognitive symptoms. The predefined sample-size re-estimation, which remains blinded to the company, was based on the cognitive domain of the clinically validated PBC-40 assessment tool. The interim analysis assessed the conditional power of the study, i.e., the likelihood of achieving statistically reliable results if the study proceeds according to the current protocol. The interim analysis was triggered by three-quarters of the intended study size having completed the treatment period in the study. The analysis concluded that no changes to the study design or target enrollment are required. The study will therefore continue as planned, with results expected in the 2nd part of 2026.

The main efficacy objective in the ongoing second part of the study is to confirm the safety profile of golexanolone and evaluate the treatment effect of the drug candidate on several potential efficacy measures, including the gold standard PBC-40 tool and the recently validated CGI-PBC tools, to inform selection of primary efficacy endpoints for the phase 3 confirmatory study.
"We are happy to receive the news that the study of golexanolone is deemed to fulfill its purpose in its current design and are looking forward to its completion later in 2026. The drug candidate addresses a significant medical need in an area with limited treatment options, giving the compound strong commercial potential," says Viktor Drvota, Chairman and CEO of Umecrine Cognition AB.

About the Phase 1b/2a study UCAB-CT-05
UCAB-CT-05 is a randomized, double-blind, placebo-controlled, two-part Phase 1b/2a study designed to evaluate the safety, pharmacokinetics, and preliminary efficacy of golexanolone in patients with primary biliary cholangitis (PBC) who experience clinically significant fatigue and cognitive symptoms. Part A (5 days, 40 mg twice daily) assessed safety and pharmacokinetics, while Part B (28 days, 40 mg or 80 mg twice daily) is evaluating efficacy using validated patient-reported and clinical measures. Key efficacy assessments include changes from baseline in the PBC-40 domains (cognition, fatigue, itch, social, emotional, and general symptoms), EQ-5D-3L, Epworth Sleepiness Scale, a cognitive test battery (PHES, RAVLT, D-KEFS), and the Clinical Global Impression of Change specific for PBC (CGI-C-PBCÔ). A pre-specified interim analysis supports adaptive sample-size re-estimation based on conditional power. The study, conducted across more than 30 European sites, continues to recruit participants following positive interim data from the first part of the study, presented at The Liver Meeting® 2024 (AASLD).

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.

For further information, please contact:

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Attachments

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