

Umechrne Cognition reports results from a Phase 1b/2a study of golexanolone in PBC-related fatigue with cognitive symptoms

STOCKHOLM – October 30, 2026. Umechrne Cognition today announced results from the second part of the Phase 1b/2a study evaluating golexanolone in patients with primary biliary cholangitis (PBC) who experience clinically significant fatigue and cognitive symptoms. Severe adverse events were infrequent and equally distributed among the three groups, with gastrointestinal symptoms among the most commonly reported adverse events, as expected. However, golexanolone did not demonstrate a statistically significant improvement versus placebo in the prespecified, hierarchically tested PBC-40 cognition endpoint, and no treatment benefit was demonstrated in the supportive fatigue analysis. Umechrne Cognition will conduct further analyses and consult external experts before deciding on the next possible steps for this indication. The company is also pursuing a Parkinson's disease program, which is supported by a separate preclinical evidence base and will continue to be evaluated independently.

The randomized, double-blind, placebo-controlled, two-part Phase 1b/2a study (UCAB-CT-05) was designed to evaluate the safety, pharmacokinetics, and preliminary efficacy of golexanolone in patients with PBC who experience clinically significant fatigue and cognitive symptoms. Part A (5 days, 40 mg or placebo twice daily) assessed safety and pharmacokinetics, while Part B (28 days, 40 mg, 80 mg or placebo twice daily) evaluated efficacy using validated patient-reported and clinical measures. The study was conducted across more than 30 European sites, where 99 patients were enrolled in Part B. Positive interim safety and pharmacokinetic data from Part A were presented at The Liver Meeting® 2024 (AASLD).

Most treatment-emergent adverse events were mild or moderate. Gastrointestinal events, including nausea, diarrhoea and headache were commonly reported. One treatment-related serious adverse event occurred in the 40 mg group. Adverse events leading to treatment discontinuation were reported in 6 of 33 patients (18.2%) receiving 40 mg BID, 3 of 32 (9.4%) receiving 80 mg BID and 2 of 34 (5.9%) receiving placebo. Substantial improvements in both fatigue and cognition were observed across all treatment groups, including a pronounced response in the placebo group. However, improvements with golexanolone did not exceed those observed with placebo.

The company will further evaluate these aggregate findings as part of its integrated clinical assessment to better understand the variability of PBC-related symptoms and their implications for future clinical development.

In addition to the efficacy results, the study generated data relevant to the evaluation of patient-reported clinical outcome measures for PBC-related fatigue and cognitive symptoms. The PBC-specific Clinical Global Impression of Change (CGI-C-PBC™) assessments showed substantial agreement between independent raters for subjective cognition (Cohen's $\kappa=0.80$) and fatigue ($\kappa=0.77$). Changes in the corresponding PBC-40 domains were correlated with clinician-rated changes in fatigue ($r=0.514$) and subjective cognition ($r=0.363$) in pooled analyses. These findings provide supportive evidence for further evaluation of the instruments' measurement properties but do not alter the negative efficacy conclusions.

The Parkinson's disease program remains supported by an independent preclinical evidence base. In addition, exploratory neurosteroid findings from the PBC study may provide complementary insights into the mechanisms targeted by golexanolone and contribute to further translational research supporting its development in Parkinson's disease.

"Part B of the study did not demonstrate the clinical efficacy signals we had hoped to establish in PBC. The study has nevertheless generated useful information on how fatigue and cognitive symptoms can be assessed, including the agreement between independent clinician ratings and their relationship to patient-reported measures. We will review the complete clinical and biomarker datasets with external experts before deciding on potential next steps for golexanolone in this indication. Our Parkinson's disease program continues to be evaluated on the basis of its distinct preclinical evidence," says Viktor Drvota, CEO and Chairman, Umechrine Cognition.

Umechrine Cognition wishes to express its sincere appreciation to the patients with PBC who took part in the study, as well as to the investigators, nurses, and study staff at participating clinics for their dedication. The complete data set will be presented at a scientific conference and submitted for publication in a peer-reviewed journal.

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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 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) evaluated 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-PBC[®]). A pre-specified interim analysis supports adaptive sample-size re-estimation based on conditional power. The study was conducted across more than 30 European sites. Positive interim safety and pharmacokinetic data from the first part of the study were presented at The Liver Meeting® 2024 (AASLD).

About Umeocrine Cognition

Umeocrine Cognition AB is developing a completely new class of drugs for the treatment of central nervous system symptoms related to disordered GABAergic signaling and neuroinflammation, which can occur as a result of underlying conditions including a range of liver diseases as well as neurodegenerative diseases, such as Parkinson's disease, and lead to severely impaired cognition and fatigue. Results from an international Phase 2 clinical study in patients with covert hepatic encephalopathy indicate that the company's most advanced drug candidate, the GABAA receptor-modulating steroid antagonist golexanolone, normalizes brain signaling and improves measures of cognition and alertness. Results from the company's Phase 2 study in patients with primary biliary cholangitis were recently reported. 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.umeocrinecognition.com.

Attachments

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