Umecrine Cognition announces top-line results from its phase 2a study with golexanolone in hepatic encephalopathy

STOCKHOLM. Umecrine Cognition AB, a Karolinska Development (Nasdaq Stockholm: KDEV) portfolio company focused on developing a new class of neurosteroid based drugs for CNS disorders, today announced results based on initial review of the topline data set from a phase 2a study of the safety, pharmacokinetics and exploratory efficacy of golexanolone (formerly GR3027) in hepatic encephalopathy.

The UCAB-CT-02 study for which the company recently announced completion of enrollment is a prospective, double-blind, randomized, placebo-controlled, multi-center phase 2a trial designed to evaluate the safety and pharmacokinetics as well as its potential effect on cognitive function of three weeks treatment with golexanolone in patients with liver cirrhosis and impaired cognitive function.

Golexanolone was generally found to be safe and well tolerated. In accordance with international guidelines, cognitive function was assessed by two or more measures; i.e., continuous reaction time (CRT) index, psychometric hepatic encephalopathy scores (PHES) and animal naming test (ANT). The central efficacy finding was that all three measures testing different aspect of cognitive function tended to improve after three weeks of treatment. However, there was no difference between golexanolone and placebo patients for any of the cognitive measures, nor was there evidence of a dose response.

"While we had hoped for a different outcome from these topline data, we look forward to analyzing the complete set of results including additional neurophysiological data from the study to further our understanding of this complex disease" said Magnus Doverskog, CEO of Umecrine Cognition. "Safe and effective treatment of overt hepatic encephalopathy (OHE) is a major unmet need, as it is a frequent and costly complication of cirrhosis, often requiring hospitalization and ICU care. Covert HE (CHE), defined by the presence of subtle cognitive defects, is potentially treatable and a major risk factor for OHE. CHE and OHE are therefore likely part of the same disease spectrum and it is plausible that treatment of CHE would reduce the risk of OHE."

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TO THE EDITORS
About the study

In UCAB-CT-02 part D, 45 patients with liver cirrhosis Child-Pugh A/B and impaired cognitive function at screening were randomized to receive 10, 40, 80 mg golexanolone, or placebo, twice daily for 21 days. Cognitive function was assessed by continuous reaction time (CRT) index, psychometric encephalopathy score (PHES), and animal naming test (ANT) in accordance with international guidelines. CRT-index ≥ 1.9 and PHES ≥ -4 was used as threshold for normal cognitive function. CRT index is a 10-minutes PC based test relaying on repeated registration of motor reaction time to auditory stimuli. The method measures and combines motor reaction speed, sustained attention, and inhibitory control, all key abilities in daily life functioning. PHES is a 20-minutes test battery consisting of five paper-pencil tests to evaluate cognitive and psychomotor processing speed and visuo-motor coordination. ANT is a 1-minute test that measures maximum number of animals listed in 1 minute. ANT is an easy and obtainable measure in development for clinical day-to-day practice for the assessment of CHE. The full data set will also contain neurophysiological assessment of brain activity (EEG), excessive daytime sleepiness (ESS), and assessment of caregiver burden and evidence of overt HE. Golexanolone treatment was well tolerated in all patients, the most commonly reported AEs were gastrointestinal disorders of mild intensity. Plasma concentrations showed that on a group level patients who received active treatment had systemic exposure of the drug candidate.

About Umecrine Cognition AB

Umecrine Cognition, a Karolinska Development (Nasdaq Stockholm: KDEV) portfolio company, is developing a potential therapy that represents a new target class relevant for several major CNS-related disorders. The primary focus is to develop a treatment for life-threatening overt hepatic encephalopathy and long-term treatment in minimal hepatic encephalopathy in patients with liver disease, a growing area with high unmet medical need. The current lack of therapeutics that directly addresses the neurocognitive signs and symptoms of hepatic encephalopathy makes a novel treatment likely to become a major contribution for the treatment of this disorder. For more information, please visit www.umecrinecognition.com.