

## Umecrine Cognition's Phase 2 study of golexanolone in Primary Biliary Cholangitis has randomized its first two patients

**STOCKHOLM – May 8, 2024.** Umecrine Cognition today announces that the first two patients have been randomized into the second part of the company's ongoing clinical Phase 1b/2 study of golexanolone. The Phase 2 study aims to evaluate early treatment efficacy signals of golexanolone on cognitive symptoms in primary biliary cholangitis, including fatigue, daytime sleepiness and cognitive function. The study is expected to include 84 patients at more than 30 clinical research centers across Europe. Based on its current timeline, the company expects to communicate top-line results in H1, 2025.

Primary biliary cholangitis (PBC) is an autoimmune disease that degrades the biliary ducts embedded in the liver, leading to liver fibrosis and cirrhosis and, if untreated, an array of symptoms that progressively decrease the patient's quality of life. While treatments exist today that reduce the progression to fibrosis and cirrhosis, none of these address the cognitive symptoms that arise from the disease. These include excessive sleepiness and fatigue, as well as other cognitive symptoms that overall lead to a significant loss in quality of life. Today, Umecrine Cognition has initiated the second part of its Phase 1b/2 study, following the recent authorization from its internal safety review committee (iSRC). This part of the study aims to evaluate early signals of treatment efficacy of golexanolone on cognitive symptoms in 84 evaluable PBC patients. The first two patients were randomized at the clinical research center at Nottingham University Hospital.

"Around one third of all patients with PBC suffer from severe fatigue and impaired cognition as part of their disease which is very debilitating in their daily life. In this study, we evaluate golexanolone's effect on patients with these symptoms and our hope is that we with this study will find a new and effective treatment that provides the opportunity for a better life for these patients," says Stephen Ryder, Professor at Nottingham University and Director of the NIHR Nottingham Clinical Research Facility.

The randomized, double-blind, placebo-controlled Phase 1b/2 study includes patients diagnosed with primary biliary cholangitis (PBC) who display clinically significant fatigue and cognitive symptoms. The participants will be randomized (1:1:1) to bidaily (BID) administration of 40 mg or 80 mg golexanolone, or placebo, for twenty-eight days. Once three-quarters of the patients have reached 28 days of treatment, an interim analysis will be performed to reassess the study's sample size. Based on its current timeline, the company expects to communicate top-line results in H1, 2025.

"There are still no treatment options available that address cognitive symptoms in PBC. We are therefore very glad to have initiated Phase 2 of our clinical study, which aims to, apart from further documenting the safety profile of golexanolone, assess the therapeutic effect of our lead drug candidate on life-disrupting cognitive symptoms in PBC. We look forward to communicating further milestone achievements in the clinical evaluation of golexanolone as the study progresses," said Anders Karlsson, CEO of Umecrine Cognition.

**For further information, please contact:**

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**About Umecrine Cognition AB**

Umecrine Cognition AB develops a completely new class of pharmaceuticals against neurological disturbances in the brain that may arise as a consequence of several underlying diseases, leading to strongly reduced cognitive functions and wakefulness. Results from an internationally recognized clinical Phase 2 study indicates that the company's most advanced drug candidate, golexanolone, normalizes the brain's signaling and improves cognition as well as wakefulness in patients diagnosed with hepatic encephalopathy. The continued drug development will initially focus on patient groups whose symptoms arise from chronic liver diseases. The mode of action is however relevant in a number of other indications. For more information, visit [www.umecrinecognition.com](http://www.umecrinecognition.com).

**Attachments**

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