Umecrine Cognition to present a novel clinically validated symptom severity scale in Primary Biliary Cholangitis at EASL 2025

STOCKHOLM – May 7, 2025. Umecrine Cognition today announces that the company will present validation and implementation data for the newly developed clinical scale CGI-S-PBC™ (Clinical Global Impression of Severity Scale for Primary Biliary Cholangitis) at the international liver meeting EASL 2025 in Amsterdam, May 7–10. The scale was designed to evaluate symptom severity, in PBC patients, that can't be measured by conventional laboratory tests. CGI-S-PBC™ was partly developed by the company and is currently being used in the ongoing Phase 2a trial of golexanolone.

Primary Biliary Cholangitis (PBC) is a chronic autoimmune cholestatic liver disease, characterized by obstructive inflammation and impaired flow in the bile ducts. PBC leads to debilitating symptoms, namely fatigue and cognitive dysfunction, or central fatigue, that cause a large negative impact on patients' daily function and quality of life. Adequate treatment of central fatigue is a critical unmet medical need in PBC, for which novel clinical scales that measure within-patient changes during symptom treatment are required. CGI-S-PBC™ is a newly developed, more objective, anchor-based clinical outcomes scale that expands on the patient-reported outcome measure PBC-40. The validation and implementation of CGI-S-PBC™ were recently documented in two separate studies and is currently being used in Umecrine Cognition's ongoing Phase IIa trial in PBC patients.

"The ongoing study by Umecrine Cognition is unique in the PBC field as it aims to target a remaining area of important unmet need, namely, to understand how we can more effectively treat symptoms like fatigue and cognitive impairment with alternative pharmacological targets and improve the quality of life", says Dr. David Jones, Coordinating Principal Investigator and Professor of Liver Immunology at Newcastle university. "However, to make the regulatory development of a new treatment modality in PBC possible, a new clinical measurement instrument for PBC, CGI-S-PBC™, was needed"

The development of CGI-S-PBC™ was led by Professor Judith Jaeger, President and Principal scientist at CognitionMetrics, LLC, together with medical advisors, and PBC Foundation UK. The scale was validated by using trained raters (n=12 blinded expert hepatologists) and measuring the scoring variability between raters. Based on statistical analyses of the variability between raters (interrater agreement), the scale was validated in six key symptom domains.

"By treating patients with central fatigue and addressing a central target for fatigue and cognitive impairment in the brain, the ongoing Phase 2 trial of golexanolone aims to translate previous robust results documented in a disease model to PBC patients", says Dr. Magnus Doverskog, SVP and CSO of Umecrine Cognition. "Further, the study will contribute to creating an effective pathway for the development and approval of new pharmaceuticals for symptom management, thus translating scientific advance and therapy development into patient benefit".

The company's abstracts will be presented at the on-site paper poster session "Immune-mediated and cholestatic disease: Clinical aspects" on Thursday, May 8th, titled: "Validation of the clinical global impression severity scale for primary biliary cholangitis: a clinical trials outcome tool" (abstract 748; i.d. THU-340), and "Implementation of a clinical global impression severity scale for primary biliary cholangitis: results of a hepatologist focused training program" (abstract #742; i.d. THU-341).

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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.

Attachments

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