Press Release

Abliva AB (publ), 556595-6538
28 June 2023 08:30:00 CEST - Lund, Sweden

Abliva announces first patient dosed in the FALCON study

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage company developing drugs for the treatment of rare and severe primary mitochondrial disease, today announced that the first patient has been dosed in the company's global Phase 2 clinical study, the FALCON study. The study will evaluate the effects of Abliva's lead drug candidate KL1333 in patients with primary mitochondrial disease.

The FALCON study, a global, Phase 2, placebo-controlled and potentially pivotal clinical study is evaluating the effects of the company's lead drug candidate, KL1333, in adult patients with mitochondrial disease suffering from chronic fatigue and muscle weakness.

Today, Dr. Rita Horvath and her team at the Department of Clinical Neurosciences, Addenbrooke's Hospital in Cambridge, UK, announced that the first patient has been dosed in the FALCON study. The study will now continue to the interim analysis which will include data from approximately 40 patients treated for up to six months. The interim analysis, a blinded analysis that will be conducted by an independent data monitoring committee (IDMC), will review the data for futility and will also provide a sample size estimation, based on the power of the two primary endpoints, that will inform the final size of the study. This informative interim analysis is expected in the first half of 2024.

The company also announced that all countries in the first wave of the study (US, UK, France, Spain, Belgium, and Denmark) have approved the study, and patients will be recruited from over 15 sites across these six countries.

"This is an important milestone for the company and for mitochondrial disease patients who suffer daily from a debilitating disease with no approved medicines. We are thankful for the strong participation from our sites and our investigators in identifying patients and we remain on track to enroll Wave 1 of the study this year with the interim analysis next year", said Ellen K. Donnelly, CEO.

Press Release

Abliva AB (publ), 556595-6538
28 June 2023 08:30:00 CEST - Lund, Sweden

For more information, please contact:

Catharina Johansson, Deputy CEO, CFO & VP Investor Relations
+46 (0)46-275 62 21, ir@abliva.com

Abliva AB (publ)
Medicon Village, SE-223 81 Lund, Sweden
Tel: +46 (0)46 275 62 20 (switchboard)
info@abliva.com, www.abliva.com

Subscribe to our news: https://abliva.com/posts/news-subscription/
Follow us on LinkedIn: https://www.linkedin.com/company/abliva
Subscribe to our YouTube channel: https://www.youtube.com/channel/UChqP7Ky5caXtp72CELhD6Mg

About the FALCON study

The FALCON study is a global, randomized, placebo-controlled, potentially registrational, clinical Phase 2 study with KL1333. Through the study, the company will evaluate the safety and efficacy of KL1333 on primary mitochondrial disease in adult patients with mitochondrial DNA mutations, with a focus on chronic fatigue and muscle weakness, which are the most common and debilitating disease expressions in these patients. The company will recruit 120 – 180 patients, in two waves, who will be given KL1333 or placebo twice daily for 12 months. An interim analysis will take place after the completion of Wave 1 and will give important statistical information on safety and powering in Wave 2.

About KL1333

KL1333 is being developed towards a treatment for a subset of adult primary mitochondrial disease patients suffering from multiple debilitating symptoms, including chronic fatigue and myopathy. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome. The KL1333 compound is a potent modulator of the cellular levels of NAD^+ and NADH, central co-enzymes in the cell's energy metabolism. In a cohort of mitochondrial disease patients in a Phase 1a/b study, the patients who received KL1333 showed both improvements in symptoms of fatigue as well as functional improvements. KL1333 is currently being evaluated in a global, potentially registrational, Phase 2 study (the FALCON study) and has received orphan drug designation in both the USA and Europe.

Press Release

Abliva AB (publ), 556595-6538
28 June 2023 08:30:00 CEST - Lund, Sweden

About primary mitochondrial disease

Primary mitochondrial disease affects the ability of cells to convert energy. The disease can manifest itself very differently depending on the organs impacted and the number of dysfunctional mitochondria in that organ. Historically viewed as clinical syndromes, our knowledge about the various mutations underlying mitochondrial disease has increased, improving our ability to identify and treat these patients. It is estimated that 125 people per million have primary mitochondrial disease. It often presents in early childhood and leads to severe symptoms, such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

Abliva – Delivering mitochondrial health

Abliva discovers and develops medicines for the treatment of mitochondrial disease. This rare and often very severe disease occurs when the cell's energy provider, the mitochondria, do not function properly. The company has prioritized two projects. KL1333, a powerful regulator of the essential co-enzymes NAD^+ and NADH, is in clinical trials. NV354, an energy replacement therapy, has completed preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

Attachments

Abliva announces first patient dosed in the FALCON study