Press Release

Abliva AB (publ), 556595-6538
20 December 2023 08:30:00 CET - Lund, Sweden

Abliva Reaches Patient Enrollment Goal for Wave 1 of 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 goal of enrolling 40 patients for Wave 1 of the ongoing, potentially registrational, Phase 2 FALCON study has been met. The interim analysis remains on track for summer 2024.

The FALCON study is a global, randomized, placebo-controlled, and potentially registrational clinical Phase 2 study evaluating the efficacy of the company's lead candidate KL1333 in adult mitochondrial disease patients experiencing fatigue and myopathy. The patients are recruited in two waves, Wave 1 and Wave 2, with an interim analysis separating the two.

Forty (40) patients have now been enrolled (randomized). The interim analysis, on track for mid-2024, will include data from the Wave 1 patients' first six months of dosing. This analysis will include a futility analysis as well as a sample size calculation for Wave 2 and will determine the final number of patients to be recruited into the full, potentially registrational, study.

"We are extremely pleased to have achieved our enrollment target for Wave 1 of the study. The rapid identification of patients by our study sites speaks to the high interest amongst patients in the FALCON study and confirms the substantial unmet medical need in primary mitochondrial disease. Our clinical sites across Europe and the US are extremely committed to the study and we all look forward to working together over the next six months to ensure delivery and quality data at the time of the interim analysis", said Dag Nesse, VP Clinical Operations.

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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 evaluates 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 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.

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 as well as Fast Track designation in the USA.

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, has entered late-stage development. NV354, an energy replacement therapy, has completed preclinical development. Abliva, based in Lund, Sweden, is listed on Nasdaq Stockholm, Sweden (ticker: ABLI).

Abliva AB (publ) - the mitochondrial medicine company. The company is listed on Nasdaq Stockholm, Small Cap, under the ticker symbol ABLI.

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Attachments

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