Press Release

Abliva AB (publ), 556595-6538
06 December 2022 09:00:00 CET - Lund, Sweden

Abliva initiates the global FALCON study with lead candidate KL1333

Abliva AB (Nasdaq Stockholm: ABLI), a clinical-stage biotech company developing medicines for the treatment of rare and severe mitochondrial diseases, today announced the start of the company's global, potentially registrational Phase 2 study with KL1333 (the FALCON study).

Abliva's lead program, KL1333, under development for the treatment of mitochondrial DNA-related primary mitochondrial diseases in adult patients suffering from debilitating fatigue and muscle weakness, has now entered into late-stage clinical development with the activation of the first site in the FALCON study. The global team will now begin screening patients into the study, a potentially registrational, Phase 2 study to evaluate the safety and efficacy of KL1333. Given a screening period of 8-12 weeks, the first patient is expected to be dosed in the first quarter of 2023.

As previously communicated, it is intended that an interim analysis of the first 40 patients will occur in late 2023/early 2024, which will provide insight into the relative chance of overall success of the study as well as inform as to the number of patients required for the remainder of the trial.

"We are thrilled today to announce the start of the FALCON study, the design of which is supported by positive Phase 1b study results, natural history data, and valuable patient input," said Magnus Hansson, CMO. "This is an important milestone for both Abliva as well as the patients, who desperately need therapies for their disease."

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About primary mitochondrial diseases

Primary mitochondrial diseases are metabolic diseases that affect the cells' ability to convert energy. The diseases can manifest 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 diseases has increased, improving our ability to identify and treat these patients. It is estimated that 125 persons per million have a primary mitochondrial disease. The diseases often present in early childhood and lead to severe symptoms such as mental retardation, fatigue, myopathy, heart failure and rhythm disturbances, diabetes, movement disorders, stroke-like episodes, and epileptic seizures.

About the FALCON study

The FALCON study is a global, randomized, placebo-controlled, potentially registrational, clinical Phase 2 study with Abliva's lead candidate KL1333. Through the study, the company will evaluate the safety and efficacy of KL1333 on chronic fatigue and muscle weakness in adult patients with mitochondrial DNA-related primary mitochondrial diseases. The company will recruit 120 – 180 patients who will be given KL1333 or placebo twice daily for 12 months. An interim analysis will take place after the completion of six months of dosing in 40 patients.

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 PMD 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.

Abliva – Delivering mitochondrial health

Abliva discovers and develops medicines for the treatment of mitochondrial diseases. These rare and often very severe diseases occur 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).

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Image Attachments

FALCON Logo RGB

Attachments

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